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IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF UTAH, CENTRAL DIVISION

JANET SCHUBERT, individually, and as personal representative on behalf of the legal heirs of Dr. William Schubert, deceased, Plaintiff, vs. GENZYME CORPORATION, a Massachusetts corporation; SANOFI, a French company; BUSINESS ENTITIES I through X; and JOHN DOE and JANE DOE, husband and wife, I through X, UNIVERSITY OF UTAH HOSPITALS AND CLINICS, a Utah entity; THE UNIVERSITY OF UTAH HEALTH SCIENCES CENTER, a Utah entity, UNIVERSITY HEALTHCARE, a Utah entity, the STATE OF UTAH, and NICOLA LONGO, an individual, Defendants.	**EXHIBIT A TO PLAINTIFF'S MOTION FOR LEAVE TO FILE AN AMENDED COMPLAINT** **FILED UNDER SEAL** Case No: 2:12-cv-00587 Judge Dale A. Kimball **(MOTION FOR LEAVE TO FILE EXHIBIT A UNDER SEAL FILED CONCURRENTLY HEREWITH)**

EXHIBIT A

FILED UNDER SEAL

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JANET SCHUBERT, individually, and as personal representative on behalf of the legal heirs of Dr. William Schubert, deceased, Plaintiff, vs. GENZYME CORPORATION, a Massachusetts corporation; BUSINESS ENTITIES I through X; and JOHN DOE and JANE DOE, husband and wife, I through X, UNIVERSITY OF UTAH HOSPITALS AND CLINICS, a Utah entity; THE UNIVERSITY OF UTAH HEALTH SCIENCES CENTER, a Utah entity, UNIVERSITY HEALTHCARE, a Utah entity, the STATE OF UTAH, Defendants.	**(PROPOSED) FOURTH AMENDED COMPLAINT AND JURY DEMAND** Case No: 2:12-cv-00587 Judge Dale A. Kimball

Plaintiff, Janet Schubert, individually as the surviving spouse of Dr. William Schubert ("Dr. Schubert" or "Decedent"), deceased, and as the personal representative of the Estate of Dr. Schubert on behalf of Decedent's heirs, hereby complains against Defendants as follows:

PARTIES, JURISDICTION, & VENUE

1. Plaintiff Janet Schubert ("Plaintiff") is a resident of Bannock County, Idaho. Plaintiff is the surviving wife of Decedent and the duly appointed personal representative of Decedent's estate. Decedent was also a resident of Bannock County, Idaho at the time of his death.

2. Plaintiff brings this case both on behalf of herself as the surviving spouse and as the duly appointed representative of Decedent's estate on behalf of and for the benefit of Decedent's legal heirs - Steve Schubert, Kathryn Schubert, Kristen Schubert, Emily Schubert, Liberty Pirrung, and Betsy Schubert, with respect to the wrongful death allegations of the complaint.

3. Defendant Genzyme Corporation ("Genzyme") is a corporation organized and existing under the laws of the State of Massachusetts, with its headquarters and principle place of business located at 500 Kendall Street, Cambridge, MA 02142, and doing business within the state of Utah and elsewhere in the United States. It produces the drug "Fabrazyme®."

4. Defendant in paragraph 3 may be referred to as the "Product Defendants" or "Genzyme."

5. Defendants the University of Utah Hospitals and Clinics, University of Utah Health Sciences Center, and University Healthcare are either political subdivisions or entities of the Defendant State of Utah (collectively "the University of Utah" or "Medical Defendants").[1]

[1] Plaintiff originally named Dr. Nicola Longo as a Defendant. On November 6, 2014, this Court entered an order dismissing Dr. Longo with prejudice, (Dkt. No. 145), based on the University of Utah and Plaintiff's stipulation that Dr. Longo was acting within the course and scope of his employment and that the jury would be instructed that the University of Utah is "responsible by

6. The Product Defendants and Medical Defendants may be collectively referred to as "Defendants".

7. Pursuant to Utah Code Ann. § 63G-7-401, Plaintiff properly served a Notice of Claim with, *inter alia*, the Attorney General's Office within one year after the claim arose.

8. Pursuant to Utah Code Ann. § 63G-7-601, Plaintiff has filed a cash undertaking in the amount of $300.

9. Pursuant to Utah Code Ann. § 78B-3-412, Plaintiff properly served a Notice of Intent to Commence Action with, *inter alia*, the Attorney General's Office within two years of when the claim arose.

10. Plaintiff has complied with all applicable aspects of the Utah Governmental Immunity Act, Utah Code Ann., 63G-7-101 *et seq*.

11. Plaintiff has also complied with all aspects of the Utah Health Care Malpractice Act, Utah Code Ann. § 78B-3-401, *et seq.*

12. This Court has subject matter jurisdiction pursuant to 28 U.S.C. §1332.

13. Venue is proper in this court pursuant to 28 U.S.C. § 1391.

GENERAL ALLEGATIONS

14. This suit arises out of a series of affirmative, negligent, and, in some cases knowing, reckless, and fraudulent acts by Genzyme in causing a shortage of the pharmaceutical drug Fabrazyme®, allocating supplies and providing information to consumers during the shortage in a way that manipulated patients into accepting ineffective treatment, and ultimately supplying an ineffective and "off label dose" of Fabrazyme® during the shortage without

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law for any breach in the standard of care" attributable to "Dr. Longo, or other University physicians" "even though the physicians are not named parties to the litigation," (Dkt. No. 144).

adequately warning of risks. These acts, which will be described more fully in the body of this Complaint, resulted in the premature and wrongful death of Dr. Schubert.

15. As more fully described herein, Genzyme's misconduct and negligent actions were motivated by its desire to preserve its market share for Fabrazyme® and to discourage patients from seeking alternative, life-preserving treatment during a period when Genzyme could not supply Fabrazyme® to the market in approved doses.

16. A contributing proximate cause of Dr. Schubert's premature death was the negligence of his treating physician, Dr. Nicola Longo ("Dr. Longo"), an employee of the University of Utah. Dr. Longo, a specialist in treating Fabry Disease, failed to advise Dr. Schubert that he was a poor risk to skip doses of Fabrazyme®. Dr. Longo also failed to study and investigate how to obtain the Fabry treatment drug Replagal® in the U.S. in response to Dr. Schubert's medical emergency. Dr. Shubert specifically asked Dr. Longo for assistance to obtain Replagal® which Dr. Longo agreed to provide. Dr. Longo then failed to timely apply for an emergency "compassionate use exemption" to obtain Replagal® for Dr. Schubert.

17. Fabrazyme® is marketed and sold by Genzyme as a life-preserving drug for patients afflicted with the genetic disorder Fabry Disease.

18. This Amended Complaint is being filed pursuant to a discovery deadline. However, a final complaint in this case cannot yet be prepared detailing all facts supporting Plaintiff's claims for several reasons.

19. First, from the outset of the Fabrazyme® shortage, and in this litigation, Genzyme has employed a strategy to keep many of its actions, decisions, and motives secret, employing overbroad designations of "confidentiality," attorney client, and work product privileges with

respect to its documents.

20. Second, since this lawsuit was filed, Genzyme has prevented access to its present and former employees outside of formal discovery.

21. Third, although the Plaintiff has issued many 30(b)(6) deposition notices and on July 30, 2014, the Court ordered these deposition to proceed, Genzyme still has not produced any of the 30(b)(6) witnesses for depositions.

22. Finally, Genzyme still has not completed its review of documents requested by Plaintiff in February, 2013. Genzyme continues to withhold several thousand documents from production, despite acknowledging that some of the withheld documents will have to be produced.

23. Accordingly, the allegations in this Amended Complaint are based upon a good faith review of Genzyme's incomplete production of documents, depositions of a small number of Genzyme employees, and publically available information. Plaintiff will seek to amend the Complaint once the remaining discovery has occurred.

24. In 2005, Dr. Schubert was diagnosed with Fabry Disease, a rare disease that is caused by a faulty or missing enzyme needed to metabolize lipids.

25. Fabry Disease is a life-threatening disease because the missing enzyme prevents certain lipids in the body from being broken down, causing lipid accumulation along the walls of blood vessels. Without treatment, the accumulation leads to reduced blood flow to the brain, heart, skin, kidneys, and nervous system, eventually resulting in the death of the patient.

26. Based on Dr. Longo's and Genzyme's recommendation that Fabrazyme® would effectively halt and/or delay progression of his cardiac-related Fabry symptoms, Dr. Schubert

began receiving therapy with Fabrazyme®.

27. Dr. Schubert followed the FDA-approved treatment schedule, receiving the full, approved dose of Fabrazyme® approximately every other week through IV therapy under the direction of his treating physician, Dr. Longo. He continued to receive these treatments until August, 2009 when, as described below, Genzyme's actions and misrepresentations during the shortage of Fabrazyme® caused Dr. Longo to prescribe, and Dr. Schubert to agree to, skip several treatments in August and September, 2009, and thereafter take reduced, diluted, "off label," and ineffective doses of Fabrazyme®.

28. As a result of his skipping doses and taking the reduced, ineffective doses, Dr. Schubert's Fabry Disease rapidly progressed, and he eventually died on March 6, 2010.

Fabrazyme® production and marketing.

29. At all times relevant hereto, Fabrazyme®, manufactured by Genzyme, was the only FDA-approved medication to treat Fabry Disease in the U.S. Fabrazyme® is a synthetic biological replacement for an otherwise natural enzyme lacking in those that suffer from Fabry Disease. Treatment with Fabrazyme® is often referred to as enzyme replacement therapy ("ERT").

30. The only FDA-approved use of Fabrazyme® is intravenous administration at a dose of 1.0 milligram of Fabrazyme® per kilogram of body weight, to be infused every two weeks ("mg/kg").

31. Treatment with Fabrazyme® is extremely expensive, typically costing over $200,000 per year per patient.

32. The annual pre-shortage retail cost of Fabrazyme® therapy for Dr. Schubert

during the years 2007-2008 ranged from approximately $330,816.36 to $417,861.74.

33. During the years 2007-2010, Dr. Schubert and his insurer were billed approximately $920,746.46 for Fabrazyme®.

34. These high costs caused Dr. Schubert to have to maintain expensive health insurance. Dr. Schubert paid approximately $17,192.16 in health care premiums for the period spanning from June 1, 2008-May 31, 2009 and $15,455.00 in premiums for the period spanning from June 1, 2009 until his death.

35. Once treatment with Fabrazyme® is initiated, it is usually prescribed for life. Therefore, a single patient and their insurer(s) will often pay millions of dollars to Genzyme to purchase Fabrazyme® over the patient's life span.

36. Thus, Genzyme had a large financial interest in maintaining its customers on Fabrazyme® and preventing customers from leaving Fabrazyme® therapy for a competitor's product. Once a patient leaves to a competitor, the patient may never return.

37. At all times relevant hereto, Fabrazyme® was marketed under the Orphan Drug Act, 21 U.S.C. § 360aa et seq., giving Genzyme a government-approved monopoly on Fabry ERT treatments in the U.S.

38. Because Orphan Drug manufacturers are granted a government-sanctioned monopoly, so long as a manufacturer continues to supply the drug to customers, federal law imposes an affirmative statutory duty on manufacturers of Orphan Drugs to ensure "the availability of sufficient quantities of the drug to meet the needs of persons with the disease or condition for which the drug was designated." 21 U.S.C. § 360cc(b).

39. A violation of this requirement enables the FDA to revoke an Orphan Drug's

exclusivity and approve another manufacturer to sell similar drugs for use in the U.S.

40. At all relevant times, at least one alternative drug, Replagal®, existed to treat Fabry Disease in most countries outside the U.S.

41. However, prior to at least October, 2009, Replagal® was not readily available in the U.S. due to the Orphan Drug monopoly protection given to Genzyme. Prior to Dr. Schubert's death, Replagal® could not be obtained in the U.S. unless a physician obtained a special exemption from the FDA.

42. Fabrazyme® is manufactured through a process called genetic recombination where Chinese hamster ovary cells are mixed together in a slurry with other stimulants to cause the cells to produce a synthetic biological equivalent of the enzyme lacking in Fabry patients. This mixture and process takes place in large tanks called bioreactors. The process takes months and, as Genzyme knew, production yields and production rates vary significantly from one bioreactor production cycle to the next. *See* GENZYME081316.[2]

43. The FDA strictly regulates production of biological drugs such as Fabrazyme®. Before a manufacturer can produce such drugs, the FDA must approve of the facility and the FDA typically conducts periodic inspections of the facility.

44. At all times relevant hereto, Genzyme manufactured Fabrazyme® at its plant in Allston, Massachusetts ("Allston plant").

45. From 2007 through approximately 2012, the Allston plant was the only Genzyme facility approved to manufacture Fabrazyme®. The Allston plant has a limited number of

[2] References to documents in this Amended Complaint are made to the bates number of the first page of the "parent" document and are meant to incorporate, through such reference, the entire document, including all "familial" documents.

bioreactors. Prior to 2007, Genzyme utilized the same bioreactors to manufacture several of its enzyme-based drugs, two of which were Fabrazyme® and Cerezyme®.

46. Genzyme was aware that the health of Fabrazyme® customers required that Genzyme maintain an adequate reserve inventory of Fabrazyme® at all times because (a) Genzyme was the only manufacturer licensed to sell Fabry ERT drugs in the U.S., (b) it takes months to manufacture a 'batch' of Fabrazyme®, (c) the yields from the reactors used to make Fabrazyme® vary significantly, (d) Genzyme only had one facility approved to produce Fabrazyme®, (e) Genzyme's facility had very limited manufacturing capacity, (f) it takes years to build a new facility with new bioreactors to thereby increase Fabrazyme® production, and (g) Fabry Disease is deadly if left un- or undertreated.

47. Genzyme also knew that the clinical efficacy of Fabrazyme® is highly dose-dependent.

48. Specifically, Genzyme knew that if Fabrazyme® purchasers were deprived of their full 1.0 mg/kg doses of Fabrazyme®, many purchasers would experience significant clinical deterioration and some would likely suffer premature death. *See, e.g.* GENZYME013854.

49. Therefore, long before the 2009 shortage, Genzyme knew that reducing dose below levels approved by the FDA in the event of a shortage was neither a safe nor appropriate option.

50. Moreover, it was not a legally authorized option, because Genzyme had only obtained approval from the FDA to sell Fabrazyme® in 1.0 mg/kg doses, the FDA had only evaluated the drug at that dose, and the product labelling for Fabrazyme® only lists the approved

dose of 1.0 mg/kg.

51. In the mid 2000's, Genzyme planned to bring, and then brought, another ERT drug called Myozyme® to market. Genzyme decided to manufacture Myozyme® at the Allston plant, placing further strain on its limited bioreactors.

52. Genzyme affirmatively decided to produce Myozyme® at the Allston plant without sufficiently increasing its ERT manufacturing capabilities. This decision significantly reduced the quantity of Fabrazyme® that Genzyme was able to manufacture.

53. Thus, by 2008, Genzyme knew that its existing reserves of Fabrazyme® inventory and capabilities to produce additional Fabrazyme® were inadequate and that supply shortages were foreseeable. *See* GENZYME638592.

54. Nevertheless, and despite having no backup supply or manufacturing capacity to produce reserve inventory, between 2007 and 2010, Genzyme affirmatively directed its sales force to aggressively market Fabrazyme®, Cerezyme®, and Myozyme® to new patients in existing and new geographic markets. These efforts led to further increases in demand for Fabrazyme® and other ERT drugs, caused further reductions to inventories of Fabrazyme®, and put ERT patients at increased risk in the event that there was any reduction or interruption in production at the Allston plant.

Genzyme's negligence in quality control and quality assurance.

55. During September and October, 2008, the FDA conducted an inspection of the Allston plant.

56. On October 10, 2008, the FDA issued a warning letter to Genzyme listing multiple quality control and system failures and other deviations from the current good

manufacturing practice ("CGMP"), including failing to demonstrate "critical aseptic connections" in the HVAC system, failing to fill vials in a qualified manner, failing to maintain surfaces of contraptions used in production of Fabrazyme® free from "roughing," failing to have a proper cleaning validation assessment of Fabrazyme®, and failing to properly document and respond to manufacturing "deviations." Publicly available at http://www.fda.gov/downloads/AboutFDA/CentersOffices/ORA/ORAElectronicReadingRoom/UCM175782.pdf.

57. This letter put Genzyme on clear notice that its quality control, quality assurance, and manufacturing systems were inadequate and needed immediate remediation.

58. Genzyme responded to the FDA on October 31, 2008, assuring the FDA that it would remedy the deficiencies. However, Genzyme then affirmatively chose to not remediate many of the deficient conditions identified by the FDA.

59. Upon information and belief, as a result of Genzyme's failures to correct problems with its CGMP processes, quality control, quality assurance, and manufacturing systems, Genzyme negligently allowed a virus, Vesivirus 2117, to contaminate Genzyme's Allston plant in the fall of 2008.

60. Genzyme informed the FDA and the public that Vesivirus 2117 was not believed to be harmful to humans, but caused a reduction in the yield of Fabrazyme® and other drugs produced in its bioreactors.

61. On February 27, 2009, the FDA wrote a second warning letter to Genzyme to advise Genzyme that it had not remediated many CGMP deficiencies identified in the FDA's October, 2008 warning letter. The February 27, 2009 letter also informed Genzyme that the

"objectionable conditions" at the Allston plant were caused by "significant" deviations from CGMP. The FDA stated that additional assurances were needed from Genzyme. Publicly available at http://www.fda.gov/ICECI/EnforcementActions/WarningLetters/ucm148998.htm.

62. Upon information and belief, Genzyme responded to the letter and assured the FDA that it would remediate the problems the FDA identified.

63. Genzyme again affirmatively chose not take all steps necessary to bring its plant into CGMP compliance and rectify the quality control, quality assurance, and manufacturing deficiencies.

64. In June, 2009, due at least in part to Genzyme's decisions to neither bring its plant into compliance with CGMP nor remediate its quality control and quality assurance deficiencies, Genzyme again negligently allowed Vesivirus 2117 to contaminate Genzyme's Allston plant.

65. In mid-June, 2009, Genzyme suspended production of Fabrazyme® and several other ERT drugs manufactured at the Allston plant due to the viral contamination in the bioreactors.

66. Due to Genzyme's announcement that it was halting production at the Allston plant, the Office of Orphan Products Development, a branch of the FDA, issued a letter to Genzyme on July 6, 2009. The July 6 letter stated, in part:

> Based on information provided by Genzyme to the review division, we understand that there is a supply shortage for Fabrazyme™ for the treatment of Fabry Disease caused by the suspension of production at your Allston Landing production site. Pursuant to 21 CFR 316.36, we ask you to provide a written detailed explanation of the measures being taken to assure the availability of sufficient quantities of drug within a reasonable time to meet the needs of patients with Fabry Disease. Holders of exclusivity for approved orphan drugs are expected to assure the availability of sufficient quantities of their orphan drug to meet the needs of patients. Failure to do so could result in the withdrawal of the drug's exclusive approval [21 CFR 316.36(b)].

GENZYME626545.

67. On July 9, 2009, Genzyme responded in writing. Genzyme's response misrepresented "assurances" to the FDA that it could continue to supply the market with sufficient quantities of Fabrazyme® to meet the needs of the persons affected with Fabry Disease. Genzyme misrepresented that the shortage of Fabrazyme® would "last approximately 4 weeks." Genzyme also attached a copy of the guidance document it had created for physicians and patients (discussed below) concerning supply management which contained the false statement that efforts to conserve supply would "be the same irrespective of geography." GENZYME626538.

68. At the time Genzyme sent the July 9, 2009 letter, Genzyme did not have a reasonable basis for assuring the FDA that it could continue to supply the market with sufficient quantities of Fabrazyme® to meet the needs of persons affected with Fabry Disease or that it could "assure" the FDA that the shortage would last approximately 4 weeks.

69. At the time Genzyme sent the July 9, 2009 letter, Genzyme knew that the shortage could persist much longer than 4 weeks for any number of reasons and that its existing inventory would only last a couple of months.

70. At or about the same time, in various press releases to financial analysts and in letters to patients and physicians, Genzyme represented that the shortage would "occur for a limited period," GENZYME000133, would be "a temporary shortage," GENZYME000250, and would "last approximately 6-8 weeks," GENZYME000026; GENZYME000193.

71. The FDA again inspected the Allston plant during the months of October and November, 2009 in connection with its review of Genzyme's CGMP violations.

72. On November 13, 2009, the FDA issued another warning letter detailing several

more deviations from CGMP it had observed during its October, 2009 re-inspection of the Allston plant. The letter noted, inter alia, Genzyme had received "many" reports of foreign particles, including glass and rubber particles, in vials; that Genzyme failed to identify the source of the contaminants; that Genzyme failed to perform necessary calibration of equipment used to manufacture the drugs; that Genzyme had failed to investigate the sources of other contaminations within the plant that had been discovered in past inspections; that there was no documentation showing that the equipment used in the manufacturing process was cleaned prior to use; and that particles were visible inside the aseptic fill rooms of the plant. The FDA concluded and advised Genzyme that the "Quality Assurance oversight of control process is insufficient." Publicly available at http://www.fda.gov/downloads/AboutFDA/CentersOffices/ORA/ORAElectronicReadingRoom/UCM191991.pdf.

73. In November, 2009, under pressure from the FDA, Genzyme shut down production at the Allston plant for a second time as a result of these ongoing CGMP deficiencies.

74. On November 11, 2009, Genzyme sent letters, signed by W. Blair Okita, Senior Vice President, Quality and Technical Operations, to physicians to let them know that "Genzyme has detected foreign particles in some products filled at the Allston" plant, including rubber, steel and other "fiber-like material." GENZYME060578.

75. However, Genzyme affirmatively decided to not inform physicians that it had to shut down its fill/finish operations to perform maintenance. Genzyme instructed its employees, if asked, to state that "Genzyme does not currently expect this to affect the availability of new supply of [] Fabrazyme®." *Id.*

76. It wasn't until December 1, 2009, in different letters, that Genzyme informed physicians and patients that "the temporary interruption of fill/finish operations for maintenance" would significantly impact and increase the duration of the supply shortage for Fabrazyme®. GENZYME019560.

77. Genzyme's repeated failures to bring its plant into compliance with CGMP caused the FDA to file a complaint for permanent injunction against Genzyme on May 24, 2010. In its lawsuit, the FDA petitioned the court to enjoin Genzyme from introducing adulterated ERT drugs, including Fabrazyme®, into the market, in violation of 21 U.S.C. § 351(a)(2)(B) (prohibiting production of drug in manner not complaint with CGMP) and 21 U.S.C. § 351 (c) (prohibiting production of drug whose "strength differs from, or its purity or quality falls below, that which it purports or is represented to possess"). *See* Complaint for Permanent Injunction, *U.S.A. v. Genzyme Corp et al*, 1:10-cv-10865, Dkt. No. 1 (D. Mass May 24, 2010).

78. The FDA's complaint alleged that its inspections "established that the drugs manufactured [are] . . . not in compliance with [CGMP]," *id.* at ¶ 10; that FDA inspectors had "documented forty-nine (49) separate deviations from CGMP," *id.* at ¶ 12, that the FDA inspections "established that the drugs manufactured by [Genzyme] are also adulterated within the meaning of 21 U.S.C. § 351(c), in that their strength differs from, or their purity or quality falls below, that which they purport are represented to possess," *id.* at ¶ 13, and that Genzyme's "noncompliance has continued despite repeated warnings from the FDA regarding their CGMP violations," *id.* at ¶ 18.

79. On the same day, May 24, 2010, the FDA and Genzyme entered into a consent decree which was approved by the Massachusetts Federal District Court on November 9, 2010.

See Consent Decree of Permanent Injunction, *U.S.A. v. Genzyme Corp*, Dkt. Nos. 2, 12 (Nov. 9, 2010). The consent decree permanently enjoined Genzyme "from directly or indirectly manufacturing, processing, packing, labelling, holding, or distributing any drugs" at the Allston plant "unless and until [Genzyme's] facilities, methods, processes and controls . . . are established, operated, and administered in conformity with CGMP" and unless and until Genzyme hired "an independent person [] to inspect the Allston Facility" to ensure compliance, *id* at ¶¶ 4(A) and (B), and from distributing an adulterated drug "within the meaning of 21 U.S.C. §§ 315(a)(2)(B) or (c)," *id.* at ¶ 19.

Competition abroad.

80. In 2009, in many countries outside the U.S., including Canada, Mexico, and the countries of Europe, a competitor's drug, Replagal®, was approved and was available as an ERT therapy to treat individuals suffering from Fabry Disease. Replagal® is another synthetic biological replacement for the enzyme lacking in those that suffer from Fabry Disease. Unlike Fabrazyme®, Replagal® is derived from human cells, whereas Fabrazyme® is derived from hamster cells.

81. Replagal® was manufactured by Shire Pharmaceuticals. Unlike Fabrazyme®, Replagal® was designed to be effective and was approved for sale at a dose of 0.2 mg/kg.

82. Genzyme's internal documents show that by 2009, Genzyme believed that Replagal® was, structurally and functionally, a biological equivalent to Fabrazyme®. GENZYME003017.

83. In countries where both Fabrazyme® and Replagal® were available, both had a significant share of the market and competition was keen.

84. In 2008 and 2009, Genzyme created documents for its marketing and sales force, instructing them that the drugs were biologically functionally equivalent, but that Fabrazyme® was a better treatment alternative because it was sold in a higher dose. *E.g. id.*

85. In fact, several of Genzyme's marketing and training documents were built around the talking point "only dose matters."

Genzyme's plan to favor foreign patients where there was market competition in the event of a product shortage.

86. In 2008 and 2009 Genzyme understood and taught its sales force that providing a full 1.0 mg/kg dose of Fabrazyme® was critical to effectively control the life-threatening progression of Fabry Disease conditions. GENZYME003017.

87. As discussed below, Genzyme also knew that many Fabry patients would suffer significant and irreversible medical decline if deprived of full dose Fabrazyme®.

88. By 2008, knowing that supplies of Fabrazyme® were tenuous, Genzyme had conducted work on a Fabrazyme® Global Contingency Plan and outlined a proposed response to a supply interruption in a document ("Contingency Plan"). GENZYME638592.

89. In the Contingency Plan, Genzyme acknowledged that Fabrazyme® was "very vulnerable" to manufacturing supply interruptions. The Contingency Plan detailed how Genzyme intended to respond to a Fabrazyme® supply disruption. *Id.*

90. The Contingency Plan also recognized that it would be necessary to make appropriate allocation decisions to preserve patient health if a supply shortage occurred.

91. The Contingency Plan further stated that, in the event of a supply interruption, "strong messaging" would be needed to influence physicians and patients to follow Genzyme's suggested allocation plan during the period of shortage. *Id.*

92. Genzyme's Contingency Plan states that in the event of a supply interruption, during the initial stages of any **interruption the "EU markets" would be "protected" with favored treatment** because of what the authors termed the **"business value"** of the European market. *Id.*

93. Under the Contingency Plan, patients in Europe would not, at the outset, be asked to make the same sacrifices in altering their treatment regimen as would U.S. patients. *Id.*

94. In other words, Genzyme's 2008 Contingency Plan revealed Genzyme's premeditated intent to give preferential treatment to European patients in the event of a supply interruption.

95. Genzyme planned to give "protection" to the European Market during the initial stages of a shortage because Genzyme knew that patients in Europe could easily switch to Replagal® while patients in the U.S. could not.

96. In fact, as detailed below, during the critical first months following the June, 2009 plant shutdown, Genzyme did "favor" the European market by creating more favorable allocation plans for European patients than for U.S. patients.

97. Therefore, Genzyme's Contingency Plan for Fabrazyme® supply interruptions, and the plan that was followed after the supply interruption was announced, as discussed below, placed concern for preservation of Genzyme's European market share over the health of U.S. patients and evinced a premeditated, knowing, and reckless disregard for the health of Genzyme's U.S. patients, including Dr. Schubert.

Genzyme's actions after confirming the virus in the Allston plant.

98. As described above, Genzyme announced the shutdown of the Allston plant on June 16, 2009.

99. By June, 2009, Genzyme knew that it had inadequate reserves of Fabrazyme® to last more than a few months and that it was uncertain how quickly the Allston plant could be fully remediated. Genzyme also knew that because the quantity of Fabrazyme® it could produce in each production cycle and the time each production cycle took were unpredictable, that even when the Allston plant resumed at full capacity, it would take months, at a minimum, to resume full production of Fabrazyme®. GENZYME174394.

100. Genzyme also knew that since 2008, it had been unable to remediate CGMP deficiencies noted by the FDA or to alter its internal quality assurance and control protocols in order to avoid future problems.

101. In fact, as described more fully below, Genzyme employees charged with evaluating the supply situation in the wake of the supply interruption informed upper management that supplies would be tenuous and unstable for **several years** due to inadequate manufacturing capacity and the lack of inventory. This information was confirmed in internal emails. *E.g.* GENZYME004750. This information was knowingly and fraudulently concealed from physicians, patients, the FDA and the public.

102. Therefore, in late June and early July, 2009, Genzyme knew that it could not reasonably "assure the availability of sufficient quantities of the drug to meet the needs of persons with the disease or condition for which the drug was designated," and that a shortage of Fabrazyme® was imminent.

103. However, Genzyme affirmatively misrepresented in June, 2009 press releases that Genzyme was "confident . . . in [their] ability to resolve the issue affecting the [Alston] plaint" and that "[t]he impact will be temporary." GENZYME000133. Genzyme further misrepresented that "the period of constraint" would only "last approximately 6-8 weeks." GENZYME000136.

104. Genzyme's projections in June – September, 2009 that the shortage would be 6 – 8 weeks were completely untenable.

105. At a minimum, Genzyme lacked sufficient data to make such a "confident" prediction as to the length of the shortage.

106. Shortly after Genzyme announced the Allston plant's closure, Genzyme learned that financial analysts were predicting that if the shortage was protracted, Genzyme could lose around $100,000,000 in annual sales revenues, over $30,000,000 of which were related to Fabrazyme® sales. GENZYME048765.

107. The financial analyst reports were quickly and widely circulated to many, if not all, of the key Genzyme management decision makers charged with formulating Genzyme's response to the impending shortage and drafting communications to the FDA, Fabrazyme® patients, physicians, advocacy groups, and the media. *E.g. id.*

108. Genzyme knew that to maintain its Orphan Drug monopoly in the U.S. and avoid losing Fabrazyme® patients elsewhere to Replagal®, it would have to assure the FDA and the community of Fabrazyme® users that any supply shortage would be very brief. Otherwise, as Genzyme knew, the FDA had authority to revoke Orphan Drug protection for Fabrazyme® and would likely work with Shire Pharmaceutical to bring Replagal® to the U.S. Market.

109. Genzyme also was aware that because Fabrazyme® and Replagal® were essentially biologically identical, once a patient switched to treatment with Replagal®, Genzyme might lose that patient's business forever.

110. Genzyme therefore proceeded to provide false, misleading and or groundless assurances in ongoing public announcements throughout the remainder of 2009, that (1) Fabrazyme® would only be in short supply for very short period, ranging from 6-8 weeks to a few more months, (2) problems with the Allston plant and bioreactors were being swiftly and effectively remedied, and (3) full production would soon resume. *E.g.* GENZYME000026 (June 19, 2009 supply update website post stating plant closed to "sanitize the Allston facility" that the shortage would "last approximately 6-8 weeks," and that "inventories will stabilize by the end of 2009"); GENZYME000250 (July 2, 2009 letter to patients and physicians stating production was halted "to allow for sanitization of the entire facility" and that the shortage would be "temporary"); GENZYME000139 (July 22, 2009 article following Genzyme announcement that included statement that "Genzyme has now completed the sanitization and is on-track to resume production at Allston this month"); GENZYME000045 (Sept. 1, 2009 supply update website post stating "both Fabrazyme® bioreactors . . . are up and running" and that "new inventory [] is expected to become available during the November/December timeframe"); and GENZYME046651 (September 24 letter to patients and physicians stating production was halted "to allow Genzyme to complete a comprehensive sanitization process" and that newly produced vials of Fabrazyme® "are now expected to be available in the first quarter of 2010" to "help allow patients to resume normal dosing.").

111. These communications were made so that Genzyme would not lose its Orphan Drug monopoly in the U.S. and could influence patients and physicians in the U.S. to accept doses in a manner that allowed Genzyme to favor foreign markets, especially the European market, where Replagal® could be obtained.

112. In contrast to Genzyme's representations during June – September, 2009 that the shortage would last only 6-8 weeks and would be over by December, 2009, the Fabrazyme® shortage lasted nearly three years. Full "label" dosing of Fabrazyme® at 1.0 mg/kg was never restored for Dr. Schubert and was not restored for many surviving Fabry patients until 2012.

Genzyme's initial plan to preserve its monopoly in the U.S. and its share of the European market.

113. Immediately after its June 16, 2009 announcement, Genzyme began creating a plan, consistent with the 2008 Contingency Plan, that favored European patients with a better supply of Fabrazyme® than U.S. patients were allocated so that European patients would not be as likely to switch to alternative treatments, especially Replagal®, thereby "protect[ing]" the European market and its "business value."

114. Pharmaceutical drugs, including Fabrazyme® and Replagal®, are regulated in Europe by the European Medicines Agencies ("EMA"), an entity with functions similar to the U.S. FDA.

115. Within ten days of announcing the plant shutdown, Genzyme affirmatively provided recommendations to the EMA and a European advocacy group ("EU FSWG") concerning how Fabrazyme® would be supplied to patients during the shortage. *See* GENZYME174394; GENZYME055789.

116. In these late June – early July, 2009 communications, Genzyme indicated that in the European countries, only adult female patients without clinically significant symptoms would receive doses lower than 1.0 mg/kg and that adult males should not lower their dose as a result of the announced shortage. Genzyme also gave the EMA specific criteria by which physicians could evaluate whether patients had clinically significant symptoms ("June E.U. guidance"). *See* GENZYME174394; GENZYME055789.

117. Genzyme was aware of medical evidence to support these European recommendations—specifically, that female Fabry patients respond differently to a reduction of the relevant enzyme absent in Fabry patients, making them less likely to become rapidly affected by an interruption in therapy. *E.g.* GENZYME174394.

118. However, Genzyme was concerned for business reasons that if patients in the U.S. received the same recommendations as European patients, supplies of Fabrazyme® would quickly be exhausted around the world, including Europe.

119. This would cause Genzyme to lose market share as predicted by the financial analysts. Many European physicians and patients would switch to the approved dose of Replagal® over an unapproved, reduced dose of Fabrazyme®.

120. This would also cause U.S. physicians and patients to lobby for abolition of Fabrazyme's® protected Orphan Drug status so they could more easily acquire Replagal®.

121. Thus, at the same time Genzyme proposed a medical plan to protect European male patients and European patients with severe clinical symptoms, Genzyme recommended a discriminatory plan for U.S. Fabry patients.

122. On June 27, 2009, Genzyme proposed an allocation and communications plan for U.S. Fabry patients designed to cause every adult patient in the U.S. to skip doses between July and October, 2009, in order to conserve supplies. GENZYME046901.

123. Unlike in Europe, all adults in the U.S., including males and all adults with clinically significant symptoms of Fabry Disease, were to join in skipping doses.

124. In order to fulfill Genzyme's need of having "strong messaging" to ensure U.S. physicians and patients complied with this plan, *see* ¶ 91, on June 27, 2009, Genzyme arranged to meet with a group of physicians and patient advocates, known as the U.S. Fabry Stakeholders Working Group ("US FSWG") to present Genzyme's plan for conserving Fabrazyme® in the U.S. and to obtain the group's endorsement. A number of Genzyme employees, including Dr. Gruskin, attended the meeting and provided information to the US FSWG. *See id.*

125. Upon information and belief, Genzyme organized, provided logistics to, funded meetings of, and supplied "all" necessary information to, the US FSWG.

126. Based upon review of the US FSWG's initial recommendation and materials presented to the US FSWG, Genzyme affirmatively and falsely represented to the US FSWG that its guidance and recommendations were and would be "designed to minimize risk for patients" and would "be the same irrespective of geography." *Id.*

127. However, this was not true since, at the same time, Genzyme presented a different and more favorable plan for European patients to the EMA and the EU FSWG.

128. In order to convince the US FSWG to go along with its discriminatory "dose skipping" plan, Genzyme also provided unsupported and false "forecasting" that predicted normal supply would resume by fall, 2009. *See id.*

129. As detailed above, at the time of this "forecasting," Genzyme had insufficient facts to make such predictions credible. No one outside Genzyme knew, or could have known, such was the case.

130. Genzyme also affirmatively decided to not tell the US FSWG that its plans to clean and repair parts of the Allston plant did not include overhaul of "fill/finish" operations noted by the FDA as being out of compliance at the Allston plant.

131. In order to convince the US FSWG to approve Genzyme's plan for US patients, Genzyme affirmatively told the US FSWG, and later Fabry patients and physicians, that running completely out of supplies of Fabrazyme® could only be avoided by having most U.S. adult patients, including males, skip 2 doses between July and the end of September, 2009. Genzyme misrepresented in these communications that it was likely that if patients complied with Genzyme's "dose skipping plan," a complete depletion of supplies of Fabrazyme®, or a "stock out," would be avoided.

132. A short time later, Dr. Gruskin, then Genzyme's Global Medical Director, sent an email to another Genzyme employee who attended the meeting, asking "Did we lie to the fswg?" The email response from his colleague, John King, Marketing Director of Fabrazyme®, stated "We are the only ones who didn't" lie. GENZYME047527.

133. Due to Genzyme's false representations that alteration in dosing for Fabrazyme® would only be necessary for a few months, on June 27, 2009 the US FSWG endorsed Genzyme's allocation and dose skipping plan and endorsed Genzyme's decision not to create criteria to supply full dose Fabrazyme® during the shortage to the patients with the greatest medical need for Fabrazyme®.

134. The US FSWG endorsement influenced Dr. Longo, Dr. Schubert and other patients to follow the Genzyme's dose skipping plan.

135. Genzyme then prepared "Dear Dr." and "Dear Patient" letters, issued on or about July 1, 2009 and July 7, 2009, respectively, detailing its dose-skipping plan. Genzyme created these documents and made prominent reference to the "FSWG recommendations" to ensure it had sufficiently "strong messaging" so that U.S. patients and physicians, including Dr. Schubert and Dr. Longo, would agree to skip doses from July through September, 2009. These letters included copies of the US FSWG "guidance document." GENZYME001320, GENZYME041153. Dr. Longo and Dr. Schubert relied on these representations in making their decision for Dr. Schubert to skip doses of Fabrazyme® in August and September, 2009 and to not seek alternatives.

136. In order to ensure maximum compliance by patients and physicians with its dose skipping plan, Genzyme then created lists of the 50 physicians with the most Fabrazyme® patients and instructed its field employees who worked with the physicians to regularly contact and monitor each prescribing physician to ensure that Genzyme's dose skipping proposals were communicated to clients and were followed. GENZYME021354.

137. In addition to communications explained above, to encourage compliance with its dose skipping plan, throughout July and August, 2009, Genzyme frequently posted "updates" on its supply website portraying a consistent message that everything was on track an in accordance with their initial "projections." On July 14, 2009, Genzyme posted that it the sanitization "process was completed" and that production of Fabrazyme® was "expected to resume by the end of this month as planned." GENZYME000034. On July 22, 2009, through a press release

and website post, Genzyme again confirmed it was "on-track." GENZYME000035. On August 7, 2009, Genzyme posted an update on "dose conservation measures," stating that "we still need this effort to continue with a high level of participation" and that Genzyme "strongly encourage[d]" patients to skip doses. GENZYME000038. On September 1, 2009, Genzyme posted another update stating that "it remains critically important for the patients who have not yet done so to miss the equivalent of two infusions" and that "both Fabrazyme® bioreactors . . . are up and running." GENZYME000045.

138. As explained above, Genzyme used these communications to influence, and in some cases pressure, physicians into compliance with its plan to have all U.S. adult patients skip dose regardless of their gender and medical status.

139. In July 2009, Genzyme's Clinical Science Associate, Keith Butler, contacted Dr. Longo to ensure he was complying with Genzyme's dose-skipping plan to have his all of his 11 Fabry patients, including Dr. Schubert, skip 2 doses before the end of September. GENZYME071228.

140. In July and August, 2009 Genzyme affirmatively chose not to timely inform its U.S. Clinical Science Associates that (a) the Fabrazyme® shortage was likely to last much longer than 6-8 weeks and (b) that many European patients were being treated differently than U.S. patients, based on an evaluation of which patients were most likely to be at risk of deterioration if they interrupted or reduced their Fabrazyme® therapy.

141. On September 8, 2009, Genzyme hosted a "town hall" where patients and physicians could call in and listen to updates and questions about the supply of Fabrazyme®. On the call, Genzyme employees explained that a goal in making allocations was that there would be

no distinction based on country, that the process from starting a bioreactor to finalized product ready to ship was 3-4 months, and continued to encourage all to follow Genzyme's dose skipping plan. GENZYME042842.

142. Dr. Schubert was not an appropriate candidate to skip doses because by 2009 he had a history of cardiac disease, arrhythmia, and left ventricular enlargement. However, he was never advised by Genzyme or Dr. Longo that he was at higher medical risk than most patients to decline and that dose skipping was not medically appropriate for him.

143. Nevertheless, due to the misrepresentations as alleged above, Dr. Schubert was advised to skip doses by Dr. Longo and did so.

Relevant medical literature and clinical studies.

144. Genzyme also created and followed a policy whereby market concerns caused it to mislead U.S. physicians and patients in regards to communications of material facts needed to make good medical decisions during the shortage.

145. Prior to the shortage, there were only two peer-reviewed and published patient studies studying the effect of Fabrazyme® treatment at doses lower than the full FDA-approved dose. One article's principal author was Vedder and the other's was Lubanda.

146. Genzyme knew that the Vedder study, which was published in 2007, was the only study evaluating the **clinical outcomes** of patients on reduced doses. Genzyme knew that the Vedder study revealed that a lower dose of Fabrazyme® (0.2 mg/kg) was not clinically efficacious for a large number of subjects enrolled in the study and that "no reduction in left ventricular mass or other disease parameters" was observed after nearly 2 years of treatment on the reduced dose. The Vedder study also showed that when study subjects who deteriorated on

the reduced dose were switched back to the full FDA-approved dose later, the full dose failed to stop "further progression of the disease."

147. On the other hand, the Lubanda study, published in 2009, only evaluated the effect of a lower dose of Fabrazyme® (0.3 mg/kg) on measurable biomarkers that can be evaluated by lab testing of subjects. The study found that some patients taking the reduced dose had a change in biomarker levels and some did not.

148. Of critical import, Genzyme knew that the studied biomarker had not then, and still has not, been proven to be correlated with clinical outcomes. In other words, there is no proof that Fabrazyme® in such low doses forestalls or delays the progression of cardiac or other diseases caused by the enzyme deficiency in Fabry Disease patients.

149. Additionally, the authors of the Lubanda study expressly stated in the published study paper that "the small sample size together with the short duration of this exploratory study did not permit analyses of clinical outcomes."

150. Dr. Gruskin recently confirmed during his deposition that Genzyme knows that there is no proven correlation between biomarker readings and clinical results.[3]

[3] Dr Gruskin testified that Genzyme knows that there is no proven correlation between biomarker readings and clinical results.

> Q. Well, I'm just giving you a proffer of what I believe he said, but the question is whether you agree or disagree that biomarker levels – measured biomarker levels poorly correlate with clinical outcome?
> A. No. I wouldn't say that. I would say that it has not been proven or demonstrated clinically that there is a correlation between biomarkers in clinical outcomes; not that there's a poor correlation.

Deposition of Daniel Gruskin at 30:19–31:4 (Oct. 8, 2014).

151. Nevertheless, when the 2009 supply interruption was announced, in "town hall" meetings and other communications to doctors and patients that were issued by Genzyme, Genzyme directed physicians and patients only to the 2009 study by Lubanda. Genzyme affirmatively chose to not even mention the 2007 Vedder study.

152. Genzyme also referred only to the Lubanda study in "talking points" Genzyme provided to its clinical representatives for discussion with physicians and patients during the shortage. *E.g.* GENZYME081280; GENZYME048031.

153. During all these communications, Genzyme knew that the Lubanda study could easily be misread, especially by a patient, as suggesting that a 0.3 mg/kg dose of Fabrazyme® had been shown to be, or was likely to be, clinically efficacious for many patients.

In fact, Dr. Gruskin also testified that Genzyme is aware of no clinically validated study confirming that Fabrazyme® is clinically efficacious to prevent or forestall Fabry Disease symptoms, even at a full dose.

> Q. It's your belief that if Fabrazyme® is started before the point of no return it would prevent clinical manifestations of disease?
> A. Yes, but again, that's a belief that has not yet been verified by clinical trials.
> Q. So if the question was based upon all the evidence that you are aware of, the medical evidence, has Fabrazyme® been proven to have clinical benefit to patients, what's your answer?
> A. My answer is Fabrazyme® has not been definitively proven to prevent clinical events.

Id. at 63:19–64:7.

> Q. Is there a category of patients you can say We have the medical evidence to show that for this category of patients the drug Fabrazyme® will, in fact, change clinical outcome?
> A. No. No. . . .

Id. at 69:11–16.

154. Dr. Gruskin also confirmed that in 2009, Genzyme had not studied, and had no validated research evaluating, how quickly, rapidly, or severely skipping doses of Fabrazyme® would affect the health of Fabry patients.

155. Without these material facts, the US FSWG and prescribing physicians, such as Dr. Longo, were not able to make fully informed and appropriate medical decision with patients about whether to participate in Genzyme's dose skipping plan at the start of the shortage.

156. Dr. Longo and Dr. Schubert relied on Genzyme's misleading, inaccurate, and false representations concerning the shortage, in making decisions to have Dr. Schubert skip doses and later take Fabrazyme® in the "off label" manner conveyed by Genzyme.

157. Genzyme knew of, but chose not to disclose, material facts concerning the medical risks of dose skipping and taking reduced "off label" doses of Fabrazyme®.

158. Specifically, Genzyme was aware that male patients, especially patients like Dr. Schubert with advanced clinical conditions, including atrial fibrillation, left ventricular enlargement, and cardiomyopathy, were not appropriate candidates to skip doses.

159. In fact, this information was the basis for Genzyme's June, 2009 recommendations to the EMA and EU FSWG that males and patients with clinically significant symptoms be protected from initial supply disruptions.

160. However, Dr. Schubert and Dr. Longo were not informed of these facts by Genzyme.

161. Genzyme also chose not to disclose that if a patient with Dr. Schubert's degree of heart disease were to decline after skipping or reducing Fabrazyme® doses, the medical decline might be irreversible even when full dosing resumed, as detailed in the 2007 Vedder study.

162. Had Dr. Longo and Dr. Shubert been informed of these facts, Dr. Schubert would not have agreed to skip doses.

163. Additionally, had Dr. Longo and Dr. Schubert been told that the shortage would last longer than 6-8 weeks as Genzyme misrepresented, Dr. Schubert would not have agreed to skip doses.

164. Additionally, had Dr. Longo and Dr. Schubert been informed that after skipping several doses, Dr. Schubert would still be required to accept a 30% supply of Fabrazyme® for many months to follow, regardless of his state of health, Dr. Schubert would not have agreed to skip doses.

165. Almost immediately after skipping the doses, Dr. Schubert's health began to decline significantly.

Genzyme's conduct and actions into the fall of 2009.

166. By August, 2009, Genzyme's management knew that the shortage would not end by October. Genzyme delayed in communicating this to the public.

167. At this time, Genzyme then began evaluating how to address its Fabrazyme® patients once it publically announced that normal supplies would not then be restored.

168. During this time, Genzyme was still highly concerned about losing Fabry patients to Shire, manufacturer of Replagal®. *E.g.* GENZYME003150; GENZYME003366.

169. During this time, Genzyme was aware that through a "compassionate use exemption," the FDA could approve the use of non-FDA-approved drugs on emergency, investigational, and/or treatment basis. The emergency access compassionate use exemption, which allows for approval and access to drugs within days of filing an application for an

emergency compassionate use exemption, provides for the quickest access to non-approved drugs like Replagal®.

170. During this time Genzyme knew that if a patient experienced clinical decline while on the non-FDA-approved dose of Fabrazyme®, the patient could rapidly obtain Replagal® by making such application.

171. However, Genzyme knew or should have known that most patients and many physicians, including Dr. Schubert and Dr. Longo, did not know they could rapidly obtain Replagal® during the shortage by applying for an emergency compassionate use exemption with the FDA.

172. Genzyme affirmatively decided to not tell physicians and patients about the process to obtain or investigate Replagal®. Instead, Genzyme initially instructed its employees to tell physicians and patients who asked about Replagal® that "A single dose substitution for a therapeutic enzyme is not easy to accomplish and the effort for patients and physicians likely exceeds the clinical benefit of the added dose." *E.g.* GENZYME008669. Later instructions were to simply direct patients with questions to Shire's website.

173. By the start of August, 2009, Genzyme knew that restart of the Allston plant was already weeks behind its initial unrealistic schedule. However, in August Genzyme affirmatively decided not to inform doctors or patients that they were behind schedule.

174. In August and September, 2009 Dr. Schubert was first beginning to decline due to dose skipping, but he had not yet experienced grave changes in cardiac function.

175. In August, 2009 Genzyme had received complaints from its own "field" employees about Genzyme's misleading and incorrect communications about the likely duration of the shortage.

176. For example, on August 19, 2009, Ty Donovan, a Genzyme employee, sent an email to Robert Tucker, Dr. Schubert's Case Manager at Genzyme, whose duties included communicating with Dr. Longo and Dr. Schubert about matters related to the shortage. Mr. Donovan requested "feedback" about how Genzyme handled communications during the first 8 weeks of the shortage. GENZYME069468.

177. Tucker replied by email, stating:

> Seems like the information was eeked out incrementally (to staff patients and stakeholders) with the idea of not scaring patients and Genzyme hoping for and trying to will the best possible outcome in terms of shortage… Rather than dashing people's hopes over and over again we could have helped them deal with reality… I think there is a fine line between containing the message and keeping people in the dark. This was not the time for mushroom management. (Keep them in the dark and watch them grow!) **Without the complete picture, I felt like my communication with patients was inauthentic, stilted, and potentially damaging with some patients.**

Id.

178. Despite Tucker's feedback, it was not until over a month later, September 24 2009, that Genzyme announced it would not be able to produce Fabrazyme® quickly enough to meet its false June and July, 2009 projections of restoring normal supplies by the fall of 2009. *See* GENZYME046651.

179. On or about September 23, 2009 Genzyme announced that while U.S. patients had complied with its dose skipping plan, all U.S. patients would still be required to accept further dose reductions.

180. At that time, Genzyme misrepresented that full supply would likely be restored by the first quarter of 2010. *See id*.

181. Upon information and belief, Genzyme again proposed a different and more patient-favorable policy for European patients than the plan it presented for patients in the U.S.

182. Genzyme affirmatively recommended to and informed the EMA and EU FSWG that, to avoid running out of Fabrazyme®, all adult patients would initially be offered reduced doses to the equivalent of 0.3 mg/kg every other week. However, Genzyme affirmatively stated that all "[p]atients who demonstrate a deterioration of disease should reinitiate the original treatment with Fabrazyme®." ("September E.U. guidance"). *See* EMA Press Release (Sept. 25, 2009), currently publicly available at http://www.ema.europa.eu/docs/en_GB/document_library/Press_release/2009/12/WC500018408.pdf.

183. Again, Genzyme's announced plan for patients in the U.S. was significantly different than its plan for patients in Europe.

184. As it did before, Genzyme affirmatively decided to fulfill its need of "strong messaging" to ensure U.S. physician and patient compliance, *see* ¶ 91, by convening the US FSWG.

185. In September, 2009, Genzyme again provided the US FSWG with incomplete, inadequate, and false data regarding the worldwide allocation plan and current supply situation. This information was provided to obtain the US FSWG's endorsement of Genzyme's modified allocation plan for U.S. patients.

186. Genzyme officials, including Dr. Gruskin, affirmatively convened a meeting and met with the US FSWG on September 23, 2009.

187. At this meeting, Genzyme told the US FSWG that supplies would likely allow patients to return to full dosing "in first quarter 2010." Genzyme affirmatively assured the US FSWG that its projections were realistic by telling the US FSWG that its projections and recommendations were likely to not change. GENZYME042931. This statement was recklessly made and proved to be disastrously untrue.

188. Genzyme advised the US FSWG that in order to avoid running out of Fabrazyme®, all patients would need to reduce their doses to the equivalent of 0.3 mg/kg every other week through the end of 2009. *Id.*

189. However, unlike in Europe, Genzyme did not propose exceptions or a plan whereby U.S. Fabrazyme® patients declining on the reduced dose could be quickly returned to a full dose of Fabrazyme® before the effects of the decline became irreversible. *Id.*

190. Genzyme again affirmatively told the US FSWG that its guidance would "be designed to minimize risk for patients," and that "all countries and regions" would participate. *Id.*

191. Such statements were false. Genzyme did not provide a means whereby U.S. patients showing clinical decline could receive an increased dose of Fabrazyme®. This was significantly different from Genzyme's recommendations for European patients.

192. Additionally, Genzyme's supply projections to the US FSWG in September, 2009 were again not supported by a reasonable basis.

193. In September 2009, Genzyme knew, or should have known, that its Allston plant was still not in compliance with CGMP and that severe deficiencies at the Allston plant remained, thus making Genzyme's projections unsupportable.

194. At the September 2009 US FSWG meeting, Genzyme made reference to the findings of the 2009 Lubanda study, presenting it as providing some support for a dose of 0.3 mg/kg every other week as an effective dose. *Id.*

195. Upon information and belief, Genzyme did not similarly propose the 2007 Vedder study for discussion at this meeting, and the differences between what each study measured and their results were not addressed.

196. Following the September, 2009 US FSWG meeting, Genzyme again prepared "Dear Doctor" and "Dear Patient" letters, detailing its dose reduction plan, citing the US FSWG support for this plan. GENZYME046651. Genzyme created these documents to ensure it had sufficiently "strong messaging" that U.S. patients and physicians, including Dr. Schubert and Dr. Longo, would follow Genzyme's plan and drastically reduce doses. Dr. Longo and Dr. Schubert relied on these representations in making their decision for Dr. Schubert to continue treatment on the reduced dose of Fabrazyme® and to not seek alternatives.

197. In those documents, Genzyme affirmatively cited to the Lubanda study, thereby providing false and misleading reassurance to patients, that the dose of 0.3 mg/kg would be efficacious. *Id.*

198. Genzyme again affirmatively decided to not disclose the relevant data on the lack of clinical efficacy or the results of the 2007 Vedder study.

Further developments in supply delays and communications.

199. Shortly after the supply interruption was announced, Genzyme's upper management was advised that, in fact, the shortage would not likely be "temporary." A decision was made to conceal this from physicians, patients, regulatory authorities and the FSWG's.

200. On September 30, 2009, in an email chain discussing Fabrazyme® supply, Andre Richer, the liaison between the manufacturing and business units of Genzyme, confirmed this to John King, the Director of Marketing for Fabrazyme®, and Dr. Gruskin, Global Medical Affairs Director. Mr. Richer stated "I think it is critical to realize that we will live from reactor to reactor for much of 2010. We only really get comfortable from an inventory standpoint in 2011 with the start of the new 2000Ls (either June 2011 or Dec 2011 approval)." GENZYME004750.

201. The above email shows that at the time Genzyme decided to supply patients with a 0.3 mg/kg doses of Fabrazyme®, Genzyme knew that shortages in Fabrazyme® supply would likely persist for at last another two years.

202. Had this true information about the supply situation been provided to the FDA, the information would have been material to the FDA's decision on whether it would revoke Fabrazyme's® Orphan Drug status.

203. Had the true information about the supply situation been provided to the US FSWG, it would have been material to the US FSWG's recommendations to patients.

204. Had the true information about the supply situation been provided to Dr. Longo and his patient, Dr. Schubert, Dr. Schubert and/or Dr. Longo would have acted with great urgency in September, 2009 to seek alternative treatment, such as Replagal®, through a compassionate use exemption or additional Fabrazyme® through private arrangements with other patients.

205. Had this more realistic information about the supply of Fabrazyme® been provided to the Fabry community as a whole, there would have been an outcry to create a more appropriate plan, greater efforts to provide access to Replagal® for patients, and an emergency access program would have been created for patients showing clinical decline.

Genzyme's concealment of its belief of the likely medical effects of dose reduction.

206. Genzyme also affirmatively chose to conceal from these sources what Genzyme knew could happen to patients on reduced doses.

207. During the very next month, October, 2009, and while Genzyme was implementing and communicating its plan to supply all U.S. patients with 0.3 mg/kg doses of Fabrazyme® without disclosing any medical risks for patients taking the reduced dose, Genzyme was actively marketing against Australian governmental approval of a similarly reduced dose of Fabrazyme®, and warning the Australian medical authority of grave dangers to patients if the reduced dose was approved.

208. Genzyme knew that a dose of 0.3 mg/kg dose was off-label, meaning that it had not been approved by the FDA or any other country's medical agency.

209. In September or October, 2009, when it unveiled its plan to supply Fabrazyme® to all patients in 0.3 mg/kg doses, Genzyme could have, but affirmatively decided not to, petition the FDA or any other medical agency, to approve Fabrazyme® at a dose of 0.3 mg/kg. Doing so would have exposed Genzyme to governmental regulatory scrutiny of the medical efficacy and medical risks of 0.3 mg/kg doses of Fabrazyme®.

210. During July – October, 2009, the Australian medical regulatory authority was evaluating whether it could reduce the approved dose of Fabrazyme® to 0.2 mg/kg in an effort to save its citizens 80% of the enormous cost of treatment of Fabrazyme®.

211. The Australian medical authority asked Genzyme to respond as to whether it was medically safe for patients to order Fabrazyme® in a reduced dose.

212. In responding to Australia's recommendation that Fabrazyme® be approved at a dose of 0.2 mg/kg, Genzyme's senior management, including many of the same Genzyme management involved in reviewing, approving, and communicating the plan for U.S. Fabrazyme® patients to accept a reduced dose to 0.3 mg/kg, reviewed and approved Genzyme 's response to the Australian medical authority.

213. Genzyme 's response to Australian medical authorities warned that reducing dose "to 0.2 mg/kg . . . across the board would have significant clinical consequences for patients, **with the expectation that many would suffer irreversible harm as a result of insufficient dosing,**" and that "**treatment at a higher dose is necessary and may be life-saving**." In the same communication, Genzyme stated that the suggestion to "reduce the dose of Fabrazyme® to 0.2 mg/kg in all patients ignores the cumulative evidence in the extant literature" and that to believe such a reduction could occur "with little or no loss of efficacy is conjectural." GENZYME013854; GENZYME013847.

214. In the same response letter, Genzyme officials cited to the Vedder study and its conclusion that a dose of 0.2 mg/kg of Fabrazyme® was "suboptimal" and would not "elicit[] a clinically relevant response to treatment." *Id.*

215. In a related email, Genzyme senior management stated that such a "**blanket dose adjustment would be insane**." GENZYME013840.

216. Genzyme's internal drafts, comments, and final responses to the Australian medical authority demonstrates that Genzyme knew that its September, 2009 plan for dose reduction to 0.3 mg/kg in the U.S. without an option for access to additional Fabrazyme® upon early signs of decline was against "the cumulative evidence in the extant literature" and exposed U.S. Fabry patients to unwarranted medical risks.

217. Genzyme knew that receiving a full dose was especially critical for a patient like Dr. Schubert, who had significant clinical heart disease and showed symptoms of medical deterioration after dose skipping in August and September, 2009.

218. However, Genzyme made no effort to warn Dr. Schubert or Dr. Longo that patients with clinically significant symptoms or early signs of deterioration were at significant medical risk if they took 0.3 mg/kg doses.

219. Such information was critical to Dr. Longo and Dr. Schubert and would have caused Dr. Longo and Dr. Schubert to take different actions after the September, 2009 announcement of Genzyme's dose-reduction plan in the U.S.

220. Because Dr. Longo and Dr. Schubert did not receive such information in September, 2009, Dr. Longo and Dr. Schubert did not understand the extreme urgency to obtain access to other treatments, including Replagal®.

221. Had Genzyme truly wanted to create guidance that was meant to "be designed to minimize risk for patients," "be the same, irrespective of geography," and "be based on best available evidence and experience," instead of a plan based on Genzyme's desire to preserve its

market share, Genzyme would have created a plan allowing for patients such as Dr. Schubert to access additional Fabrazyme®, through an emergency access program or by facilitating knowledge of how to access Replagal®.

222. Replagal® would have been a better alternative than 0.3 mg/kg Fabrazyme®, since it had been clinically evaluated and approved for use by many government medical regulatory agencies while 0.3 mg/kg Fabrazyme® had not.

223. Alternatively, Dr. Shubert could have located Replagal® to take in addition to the reduced Fabrazyme® dose in order to receive a greater quantity of ERT treatments.

224. Dr. Longo and Dr. Schubert relied on the information provided in support of Genzyme's September dose-reduction plan. They were not warned of the true risks of dose skipping and dose reduction and were not told the true facts relating to the likely duration of shortage.

225. Therefore, Dr. Longo prescribed and Dr. Schubert accepted, a dosing regimen equal to 0.3 mg/kg for the months of October through December, 2009 as Schubert's sole treatment and did not obtain Replagal® until February 24, 2010.

226. By February 24, 2010, Dr. Schubert's cardiac deterioration caused by his Fabry Disease was grave and irreversible.

Genzyme's continued misrepresentations as to the duration of the shortage.

227. As detailed above, an October and November, 2009 inspection by the FDA resulted in findings that Genzyme had still failed to remedy CGMP deviations that the FDA had identified over a year earlier and ordered Genzyme to remediate. *See* ¶¶ 71–73, supra.

228. In November, 2009, Genzyme again suspended production at the Allston plant due to these problems.

229. Following this event, Genzyme supplied another written announcement to patients and physicians on December 1, 2009. GENZYME019560.

230. On November 29, 2009, in discussing issues related to supply, Geoffrey McDonough, a top Genzyme officer, explained to Dr. Gruskin, John King, and Ralph Kern, "…In the worst case, Genzyme anticipates resuming full supply of Fabrazyme® by the end of 2010." GENZYME073484.

231. However, Genzyme affirmatively chose to not communicate this scenario in its direct mailings to Fabrazyme® customers and physicians.

232. Instead, in Genzyme's December 1, 2009 "Dear Dr." and "Dear Patient" letters, Genzyme stated that patients would continue to receive Fabrazyme® at 0.3 mg/kg "through March of 2010," but that "Genzyme plan[ned] to resume shipping Fabrazyme® at 70 – 100% of full dose . . . beginning in the second quarter of 2010." GENZYME019560.

233. In the December 1, 2009, letters, Genzyme again decided to not include information about the treatment of patients in Europe, or about the existing medical literature which did not support the clinical efficacy of .3 mg/kg Fabrazyme® therapy. *See id.*

234. Dr. Schubert and his wife received the Dear Patient letters, including the December 1, 2009 letter and had a phone call with Genzyme's customer service representative on December 3, 2009. Dr. Longo and Dr. Schubert relied on these representations in making their decision for Dr. Schubert to continue treatment on Fabrazyme® and to not seek alternatives.

235. Revealing that Genzyme was still not being truthful in communications with its prescribing physicians and patients, on January 14, 2010, Dr. Gruskin forwarded an email stating "Enjoy! With the most recent ~~lie~~ update, I did not produce another guidance document – we just sent out letters to doctors/patients/payors saying the guidance will be continued." GENZYME035638.

236. The "~~lie~~ update" Dr. Gruskin was referring to was Genzyme's most recent false communication, projecting that full dosing would likely be restored within a few months.

237. Genzyme did not inform patients and physicians that its "prediction" was again incorrect until February 17, 2010.

Genzyme's refusal to supply emergency access Fabrazyme® to Dr. Schubert.

238. On December 3, 2009, Mrs. Schubert wrote an email to Genzyme during a "Town Hall" meeting Genzyme held that day. Mrs. Schubert advised Genzyme of the gravity of Dr. Schubert's medical situation and his decline due to the supply interruption.

239. Her email stated, in part:

> …I am watching my husband's hearing and vision change rapidly to the point the hearing aids are losing effectiveness. His vision has changed dramatically during the reduced schedule… We can no longer hang on, adjust, adapt, reduce our needs and work requirements, cut back, down size or turn down the thermostat further… We have struggled as well with constant concerns about affording the enzyme and medical insurance until Medicare is available… The health effects since the reduction are really serious, and go beyond being able to work. There are catastrophic health implications without the full dose."

240. For the next three months, Dr. Schubert, his wife, and his physician, Dr. Longo, repeatedly pleaded for Genzyme's assistance with obtaining more Fabrazyme®. However, Genzyme never provided it.

241. By December, 2009, Dr. Longo, as well as other physicians and patients, had communicated to Genzyme that it was important and necessary to have an emergency access program for patients in medical need to access full dose Fabrazyme®.

242. Despite these requests, prior to Dr. Schubert's death, Genzyme declined to establish an emergency access program for U.S. Fabry patients.

243. Additionally, Genzyme declined to establish a uniform procedure/process to quickly evaluate and respond to requests for additional Fabrazyme®.

244. These decisions led to inconsistent and inequitable results; some patients in need were denied additional Fabrazyme® while others received it. *E.g.* GENZYME019806.

245. These decisions also resulted in negligent delays in processing Dr. Longo's and Mrs. Shubert's urgent requests for additional Fabrazyme®.

246. Specifically, five months passed and Dr. Schubert's health suffered irreversible declines before Genzyme responded with an offer of doses of Fabrazyme®.

247. Dr. and/or Mrs. Schubert first contacted Dr. Longo in early October, 2009 to let him know about Dr. Schubert's declining health.

248. Due to Dr. Schubert's declining health, Dr. Longo then contacted the regional medical director of Genzyme, Tim Miller, to request additional Fabrazyme® for Dr. Schubert.

249. Genzyme affirmatively denied Dr. Longo's request, claiming there was no extra Fabrazyme®. No medical reason was given.

250. However, during late 2009 and the beginning of 2010, Genzyme granted several requests by other patients for additional Fabrazyme®, including a request from a patient with advanced cardiac involvement like Dr. Schubert. *E.g. id*.

251. On December 3, 2009, Sarah Iden, a medical affairs liaison, spoke with Dr. Schubert about the extension of the 0.3 mg/kg dose. Dr. Schubert told Sarah Iden about his continued physical decline on the reduced dose.

252. On that same day, Keith Butler reported that Dr. Longo was "not happy with the latest FZ delay" because Dr. Longo "has two patients," one of whom was Dr. Schubert, "that are not doing well at reduced doses." GENZYME036093.

253. Dr. Longo spoke with Dr. Miller on or about December 3, 2009. Dr. Longo requested additional Fabrazyme® for Dr. Schubert.

254. Genzyme again affirmatively denied Dr. Longo's request without providing a medical reason why.

255. In late December, 2009, Dr. and/or Mrs. Schubert again contacted Dr. Longo to inform him of Dr. Schubert's continued decline in health.

256. On December 22, 2009, Dr. Longo also sent an email to Tim Miller, the regional medical director of Genzyme, stating he "would like to have more infusions" for Dr. Schubert. Dr. Longo explained:

> This is a 63 year old male with the cardiac variant of Fabry Disease. He is still working full time… He has full cardiac involvement by Fabry… and cardiac failure … with ups and downs. With ERT his cardiac function has improved… Since reducing the dose of ERT, he has been unable to get to the end of the day. He had worsening of his edema and of his proteinuria that was improving on stable ERT. My concerns are that the increased demand placed on the cardiovascular system … might cause the heart to fail. I am asking whether additional doses of Fabrazyme® would be available to treat this specific patient.

GENZYME035878.

257. The December 22, 2009 email was forwarded to Dr. Gruskin. *Id.*

258. The next day, in an internal email, Dr. Gruskin told a colleague he could not grant the request. *Id.*

259. Dr. Gruskin stated in that email "And honestly, there is no evidence that FZ benefits patients at this late stage." *Id.*

260. Genzyme did not then, and has never has, disclosed to Dr. Longo, Dr. Schubert, or the U.S. Fabry community that "there is no evidence that FZ benefits" late-stage patients.

261. Genzyme has and, upon information and belief, still continues to sell monthly infusions of Fabrazyme® to patients worldwide with advance stage disease, up to the end of their lives, at a cost to the patient and/or their medical payors of approximately $20,000 per month or more.

262. Dr. Gruskin's statement that Fabrazyme® does not offer benefit to late stage Fabry Disease patient would have been material to Dr. Schubert and/or his medical payor's decision to continue to pay for reduced dose Fabrazyme® therapy.

263. Genzyme continued to bill Dr. Schubert and his insurer for monthly Fabrazyme® infusion therapy after December 22, 2009

264. Dr. Longo continued to press Dr. Schubert's urgent need for more Fabrazyme® with Genzyme employees in January, 2010.

265. As a result, Dr. Longo's December 22, 2009 email request for more Fabrazyme® was again forwarded to Dr. Gruskin on January 13, 2010. GENZYME035647.

266. Genzyme again affirmatively refused to provide additional Fabrazyme®, stating that there was "nothing we can do." No medical reasons were given to Dr. Schubert. *Id.*

267. Prior to his being hospitalized on a January 30, 2010, Mrs. Schubert again called Genzyme to request more Fabrazyme®. She spoke with Tim Miller, a regional medical director for Genzyme, and with Sarah Iden and explained that Dr. Schubert's health was rapidly declining.

268. On January 30, 2010, Dr. Schubert had to be admitted to the hospital for cardiac related symptoms for the first time since the shortage began. He was observed to sustain a transient ischemic attack, which is an event related to his Fabry Disease.

269. Dr. Schubert remained in the hospital for 2 days. At the time of his discharge it was noted "the amount of atrial fibrillation" Dr. Schubert suffered "was increasing the previous two months compared to previous several months before that." In other words, Dr. Schubert's health was declining much more rapidly on the reduced dose of Fabrazyme® than it was on the full dose of Fabrazyme®.

270. Dr. Longo and Genzyme employees were advised of this hospitalization at the time of the event. Mrs. Schubert advised Dr. Longo of the medical details and advised him that Dr. Schubert had urgent need for more Fabrazyme®.

271. On or about January 31, 2010, Mrs. Schubert called Tim Miller, a regional medical director for Genzyme, to personally plead the urgency of the situation and the need for more medication.

272. Dr. Miller acknowledged in an internal email that he spoke to Mrs. Schubert and that he told her Genzyme was "still working to find a solution." GENZYME034727.

273. Unfortunately, nothing further appears to have been done at Genzyme to "find a solution" between this phone call and February 24, 2004.

274. On or about February 12, 2010, Dr. Schubert was readmitted to the hospital. At this time, an evaluation revealed that he was now suffering acute symptoms of congestive heart failure.

275. On February 23, 2010, Dr. Longo sent another email to Dr. Miller at Genzyme, once again requesting additional Fabrazyme® for Dr. Schubert. Dr. Longo explained:

> My patient with Fabry Disease has been in the hospital on and off until now since the time you spoke with his wife. He had a stroke and the heart failure… Can we get his next two doses anticipated so we can treat him transiently every 2 weeks to see if we can get him out of the hospital?

GENZYME035117.

276. Dr. Miller forwarded this email to Dr. Gruskin. Dr. Gruskin spoke to Andre Richer and reported:

> Spoke to andre – he is cool with making a 1 time exception. To be clear, this patient will receive his allotment of 2 doses early, but will not receive another shipment til June.

Id.

277. However, because of Genzyme's negligence, Genzyme did not ship the 2 newly approved doses.

278. On March 3, 2010, Dr. Longo contacted Dr. Gruskin to find out why the approved doses had not shipped. Dr. Gruskin then finally communicated to Genzyme employees in shipping that they were to ship additional Fabrazyme® to Dr. Schubert. GENZYME035015.

279. The promised doses never shipped before Dr. Shubert died.

Dr. Longo's and the Schuberts' efforts to obtain Replagal®.

280. On October 21, 2009, Shire announced that it had begun the necessary process to make Replagal® available for treatment in the U.S. based on a treatment compassionate use exemption.

281. Thereafter, Dr. Schubert contacted Dr. Longo to request his assistance in obtaining Replagal® as soon as possible.

282. In November or early December, 2009, Dr. Longo contacted Shire to ask whether Dr. Longo could obtain approval for all of his Fabry patients to receive treatment with Replagal®.

283. Dr. Schubert called Dr. Longo on December 23, 2009. Mrs. Schubert overheard her husband's conversation with Dr. Longo. Dr. Schubert asked Dr. Longo "about the compassionate use exemption and treatment options" and asked for help obtaining Replagal® as soon as possible.

284. At this time, Dr. Longo agreed to assist in this regard and offered to contact Shire.

285. However, prior to Dr. Schubert's death, Dr. Longo failed to consult the FDA website, speak with representatives of Shire about, or otherwise research how to obtain an expedited "compassionate use" exemption based on a medical emergency.

286. Dr. Longo never investigated whether Replagal® could be quickly obtained through an emergency compassionate use exemption.

287. Therefore, prior to Dr. Schubert's death, Dr. Longo never became aware, and did not advise Dr. Schubert, that Replagal® could be obtained in a few days or weeks through an emergency compassionate use exemption.

288. Dr. Longo knew only about what is sometimes referred to as a treatment compassionate use exemption, which takes many months to process and requires a hospital to create, review, approve, and submit a clinical study protocol. Dr. Longo eventually worked on a treatment compassionate use protocol wherein he reached an agreement with Shire that the University of Utah would receive a large amount of money for each patient enrolled in the Replagal® treatment protocol at the University of Utah.

289. During the second or third week in January, 2010, Dr. Schubert again called Dr. Longo to ask about Dr. Longo's efforts to obtain Replagal®.

290. During this conversation, Dr. Longo told Dr. Schubert that he had not started the application to acquire Replagal® and that a clinical trial protocol for Replagal® would take six months. He indicated to Dr. Schubert that there were no other options to get Replagal® in the meantime.

291. In mid-February 2010, Dr. Schubert learned from another doctor that Dr. Longo was incorrect. The other doctor was able to quickly obtain Replagal® by submitting an emergency compassionate use exemption application to the FDA and Shire which was quickly granted.

292. However, due to the delay in requesting the emergency compassionate use exemption, Dr. Schubert did not have a Replagal® infusion until February 24, 2010. By then, Dr. Schubert's health had already sharply declined with severe progression of congestive heart failure.

Dr. Schubert's death.

293. Dr. Schubert died from Fabry-related complications on March 6, 2010.

294. Had Dr. Schubert not skipped doses in months of July through September, 2009, and had Dr. Schubert been provided access to a full dose of Fabrazyme® upon initial signs of clinical decline, Dr. Schubert would have lived an unknown, but assuredly longer, amount of time.

295. Dr. Tim Miller, who was Genzyme's regional medical director over the state of Utah, testified that Dr. Schubert's clinical condition would have met the medical criteria to receive full doses of Fabrazyme® under Genzyme's emergency access protocol promulgated for European patients.

296. Had Schubert been provided true information about the duration of the shortage, the medical risks of dose skipping and off label dosing, and how to access other options, Dr. Schubert could have sought full dose Fabrazyme®, he could have sought the approved dose of Replagal® before his condition deteriorated to critical, or he could have obtained and taken both drugs in sequential infusions. Such alternatives would have prolonged Dr. Schubert's life and prevented much pain and suffering.

297. Following Dr. Schubert's death, Dr. Gruskin continued to be involved in regard to Genzyme's response and communications concerning the ongoing shortage.

298. On January 18, 2011 in an email to his colleague, Andre Richer, Dr. Gruskin stated "We totally screwed the pooch and PV is to blame, although we let them do it. We sent the EMA bullshit data and then are surprised when they come up with recommendations to switch to Replagal®." GENZYME511440.

299. On February 27, 2012, Dr. Gruskin commented in a company email to a colleague in anticipation of his annual review by Genzyme management:

> They don't like it when peons like me say the truth. They tell me I have a "bad attitude" and negatively affect other people. Rogerio was really pissed at me at an offsite….I said that altho individually people are still very patient focused, the company isn't , as shown by how we managed /communicated during the shortage….."

GENZYME549334.

DAMAGES

300. As a direct and proximate result of the actions described herein, Dr. Schubert suffered severe injuries and death including, but not limited to, stroke, cerebrovascular transient ischemic events, progression of cardiac disease including cardiomyopathy, severe hypertrophic restrictive cardiomyopathy, severe low cardiac output, right ventricular and left ventricular failure, biatrial enlargement, cardiogenic shock, congestive heart failure, decreased left ventricular systolic function, severe left ventricular hypertrophy, severely enlarged right ventricle, dilated right atrium, increased left ventricular wall thickness, atrial fibrillation, tachycardia, diminished cardiac output, advanced heart failure, cardiomegaly and congestive heart failure resulting in death, progression of renal disease, abnormal liver function, sinusitis, neurological decline, discoordination, agitation, dyspnea, extreme fatigue, extreme physical pain, loss of hearing, loss of sight, double vision, abdominal pain, thickening of the gallbladder, gastrointestinal complications, severe emotional distress, anxiety, depression, weakness, general deterioration of overall health, other medical complications as the evidence may show at trial, and eventual premature death.

301. Plaintiff makes claim for premorbid pain and suffering of the decedent on behalf of the heirs of Dr. Schubert.

302. Plaintiff makes claim for the unnecessary medical expenses incurred by the Estate of Dr. Schubert, including but not limited to, unnecessary medical expense to acquire non therapeutic and non efficacious dose treatments of Fabrazyme®.

303. Plaintiff also makes claim for the medical expenses resulting from Dr. Schubert's hospitalizations between the initiation of the Fabrazyme® shortage and Dr. Schubert's death approximately eight months later. These claims include medical expenses incurred by Dr. Schubert which were paid by Blue Shield of Idaho, which has asserted a subrogation lien.

304. Ms. Schubert makes claim for her own loss of consortium damages preceding Dr. Schubert's death, for her own pain and suffering, and for damages on behalf of herself and the children of Dr. Schubert as personal representative of the Estate.

305. As a direct and proximate result of the actions described herein, Dr. Schubert and Plaintiff sustained non-economic damages including, without limitation, pain and suffering, severe emotional distress and mental anguish, loss of care, loss of companionship, and other non-economic damages recoverable by law as the evidence may show at trial.

306. As a direct and proximate result of the actions described herein, Plaintiff will continue to sustain non-economic damages including, without limitation, pain and suffering, severe emotional distress and mental anguish, loss of care, loss of companionship, and other non-economic damages recoverable by law as the evidence may show at trial.

307. As a direct and proximate result of the actions described herein, Dr. Schubert and Plaintiff sustained economic damages including, without limitation, reasonable and necessary medical expenses, funeral and burial expenses, wage loss, loss of household services, loss of

economic support, and other economic damages recoverable by law as the evidence may show at trial.

308. As a direct and proximate result of the actions described herein, Dr. Schubert and Plaintiff suffered consequential damages including, but not limited to, the amounts paid for Fabrazyme® therapy from August, 2009 through March 6, 2010 or, in the alternative, the amounts paid for Fabrazyme® therapy from 2005 through March 6, 2010, the increased cost of medical insurance to cover medical costs associated with Fabrazyme® treatment, and other consequential damages as the evidence may show at trial.

FIRST CLAIM FOR RELIEF
(Negligence v. Product Defendants)

309. Plaintiff incorporates all preceding paragraphs and further alleges the following:

310. Fabrazyme® was designed, manufactured, sold, promoted, tested, and/or distributed through the stream of commerce by the Product Defendants.

311. Because Product Defendants affirmatively decided to sell patients, including Dr. Schubert, Fabrazyme® and determined to keep supplying Fabrazyme® after the supply interruption while causing patients to skip doses and later sold Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to Plaintiff and Dr. Schubert with respect to its inventory planning, production planning, marketing, manufacturing, quality control and quality assurance, allocation planning after the shortage, and sale, distribution, and customer service.

312. Additionally, because Product Defendants affirmatively chose to be the sole manufacturer allowed to produce and distribute an ERT treatment therapy for Fabry patients and continued to push to maintain itself as the sole manufacture once the shortage of Fabrazyme®

was imminent, which treatment Product Defendants knew was a necessary and even lifesaving drug, and because of Product Defendants chose to continue to manufacture, sell, promote, test, and/or distribute Fabrazyme® to Dr. Schubert in quantities of 0.3 mg/kg, which was not an FDA-approved "label" dose, and because Product Defendants made repeated affirmative representations during the relevant shortage, Product Defendants owed an affirmative duty to patients and physicians, including Dr. Schubert, Plaintiff, and Dr. Longo to use reasonable care so that patients would not be harmed by taking reduced, off label doses of Fabrazyme® in lieu of seeking additional or other ERT therapies options.

313. Product Defendants breached this duty by affirmative acts detailed in paragraphs 1–308, including, but not limited to, these events occurring prior to the announcement of the shortage:

a. Negligently deciding not to increase manufacturing capability for Fabrazyme® after the introduction of a new drug and expansion of the ERT drug market between 2006-2009, despite knowing that Fabrazyme® supplies would be inadequate in the foreseeable event of any supply interruption, which actions put Fabry patients at medical risk of deterioration or death;

b. Negligently maintaining their facility out of compliance with CGMP;

c. Negligently performing inadequate maintenance of the Allston plant, bioreactors, and filling equipment despite repeated notices and warning letters from the FDA;

d. Negligently maintaining inadequate aseptic conditions in required

areas of the Allston plant, thereby exposing the plant to contaminants and increasing the likelihood of a contamination;

e. Negligently refusing to correct inadequate quality control and quality assurance procedures;

f. Negligently refusing to properly address the deficiencies and deviations from CGMP noted by the FDA in October, 2008, February, 2009, and November, 2009 and at other times in a timely manner, which ultimately required closure of the Allston plant and the resultant supply interruption and shortage of Fabrazyme;

g. Negligently creating an allocation plan which provided favored treatment to EU patients at the expense of U.S. patients, without medical justification;

h. Negligently creating an allocation plan which did not include an emergency access plan for patients whose health declined on reduced or skipped dose;

i. Negligently creating an allocation plan that did not provide any protocol to quickly respond to patient or physician requests for additional Fabrazyme® if there was a medical need; and

j. Other acts of negligence as plead, or which may be discovered during this case.

314. Once the supply interruption was announced and Product Defendants affirmatively chose to continue to manufacture, sell, promote, test, and distribute Fabrazyme® in

reduced, off label doses, Product Defendants' negligent acts included but were not limited to:

a. Negligently failing to fully remediate the CGMP deficiencies when the Allston plant was closed in June, 2009, which required a second plant closure;

b. Negligently creating an inequitable, unequal, and medically inappropriate allocation plan for U.S. patients with clinically significant symptoms of Fabry such as Dr. Schubert;

c. Negligently providing improper, incomplete, and misleading information to physicians and patients about the length of the shortage, the medical risks of adhering to the recommendations, treatment alternatives, and Product Defendants' response to the shortage, especially from July, 2009 through February, 2010, and all other times there between where Product Defendants communicated with Dr. Longo, Dr. Schubert, Mrs. Shubert and others who influenced treatment decisions for Dr. Schubert;

d. Negligently designing a plan, beginning in 2008, whereby Product Defendants would base its response to a shortage of Fabrazyme® in a manner that would favor the European market at the expense of others and then relying on the principles of that plan during the actual shortage;

e. Negligently favoring European patients over U.S. patients, including Dr. Schubert and Plaintiff, with respect to allocation decisions during the early stages of the shortage;

f. Negligently including male patients with clinically significant cardiac disease, like Dr. Schubert, in its "dose skipping" plan;

g. Negligently including male patients with clinically significant cardiac disease, like Dr. Schubert, as appropriate patients to receive the equivalent of 0.3 mg/kg doses of Fabrazyme;

h. Negligently refusing to create a means whereby patients exhibiting clinical decline, like Dr. Schubert, could apply based on established criterial to obtain additional Fabrazyme® as was done in Europe;

i. Negligently refusing to create a plan to efficiently process emergency medical requests for additional Fabrazyme® so that decisions would be uniform, processed timely, and based on justifiable medical criteria;

j. Negligently contacting patients and physicians, like Dr. Schubert and Dr. Longo, in July 2009, and informing them that their failure to comply with Product Defendants' plan would result in a complete depletion of Fabrazyme® supplies and thereby harm many people, but that by complying with Product Defendants' plan it was likely that full supply would be restored by the end of 2009;

k. Negligently and recklessly making forecast projections and/or communicating to patients proper information about the anticipated length of the shortage;

l. Concealing important medical risks to patients to whom Product Defendants intended to offer a reduced dose and/or supply of Fabrazyme®, while simultaneously highlighting these serious medical risks in non-public communications to Australian medical regulators and others;

m. Despite repeated calls to do so, refusing create a uniform system in the U.S. to respond to requests from patients and physicians, like Dr. Schubert, Plaintiff, and Dr. Longo, for additional Fabrazyme® for patients who suffered serious medical decline while skipping or taking reduced doses;

n. Refusing requests to supply additional Fabrazyme® to Dr. Schubert while some other patients' requests for additional Fabrazyme® were approved;

o. Unreasonably delaying action on Dr. Schubert's requests for additional Fabrazyme;

p. Providing and encouraging the use of Fabrazyme® in lower doses than was approve by the FDA;

q. Making efforts to either block or discourage those taking Fabrazyme® from switching to Replagal;

r. Providing incomplete, inadequate, and misleading warnings of the known or knowable dangers involved in the use of Fabrazyme®, especially at a reduced dose;

s. Communicating false and misleading information to the FDA in order to retain a commercial monopoly on Fabry ERT therapy for U.S. patients during the shortage;

t. Failing to adequately inform patients unable to access full dose Fabrazyme® of their treatment options and how to access them; and

u. Otherwise failing to exercise the care and caution that a

reasonable, careful, and prudent entity would have or should have exercised under the circumstances.

315. Product Defendants' duties also included, but were not limited to, a duty to warn of medical risks and limitations related to Fabrazyme® therapy, gleaned from Product Defendants' internal assessments, evaluations, clinical trials, and review of independent trials over a period of many years. Such warnings should have disclosed, and included, but were not limited to: that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, such decline would often become irreversible even if full dose was later restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

316. Product Defendants, individually and/or collectively, had a significant financial incentive to suppress, misrepresent, and/or conceal any potential dangers or risks associated with the lower dose of Fabrazyme®.

317. By virtue of their negligence, the Product Defendants are liable for the severe injuries, death, and conditions as set forth herein.

318. As a direct and proximate result of the aforementioned injuries, Plaintiff and Dr. Schubert suffered the damages described herein.

319. The Product Defendants acted together, with a common profit motive, in failing to

adequately or appropriately disclose material information relating to the production problems and ineffective lower doses.

320. At all relevant times hereto, the Product Defendants' conduct as set forth herein was done with conscious indifference and knowing and reckless disregard for the safety and well-being of individuals, including Dr. Schubert, and such conduct justifies the imposition of punitive damages against all of the Product Defendants.

321. As a result of these injuries, death, and conditions, Plaintiff has suffered damages as set forth herein.

SECOND CLAIM FOR RELIEF
(Strict Liability v. Product Defendants)

322. Plaintiff incorporates all preceding paragraphs and further alleges the following:

323. The Product Defendants are liable under the theory of strict liability under the common law and/or Utah Product Liability Act. The Product Defendants placed Fabrazyme® into the stream of commerce in a manner that was unreasonably dangerous, without limitation, as follows: defects in the design; defects in the manufacture; defects in the warnings; defects in the design of 0.3mg/kg Fabrazyme®, and/or other defects that may later be revealed during discovery.

324. Defendants marketed and sold to patients, including Dr. Schubert, Fabrazyme® for off-label use in dose-skipping and 0.3 mg/kg quantities, which doses were never approved by the FDA nor clinically evaluated by the FDA. Further, Product Defendants could have petitioned the FDA for supplemental labeling for Fabrazyme® in 0.3mg/kg quantities, infused bi-monthly, but did not. Accordingly, Product Defendants are not subject to the protection of comment K to Restatement (Second) of Torts 402A, as adopted by the Utah Supreme Court in

Grundberg v. Upjohn Co., 813 P.2d 89 (Utah 1991).

325. Without limitation, the design of the Fabrazyme® in "off label" dosing quantities was defective and unreasonably dangerous because:

a. At quantities lower than approved by the FDA, Fabrazyme® was not proven to have a clinical benefit for patients like, and including, Dr. Schubert at reduced doses;

b. At quantities lower than approved by the FDA, Fabrazyme® was an off-label drug and was not fit, suitable, or safe for its intended purpose;

c. At quantities lower than approved by the FDA, Fabrazyme® did not prevent or forestall progression of Fabry-related symptoms, including, but not limited to, cardiac and cerebrovascular disease;

d. At such dose, Dr. Schubert suffered a rapid and accelerated decline in health, and died prematurely, and;

e. In all other manners alleged herein.

326. Such design caused Fabrazyme® to be dangerously defective, and such defect existed at the time it left the Product Defendants' possession.

327. Without limitation, Fabrazyme® was manufactured in a manner that was unreasonably dangerous and defective because:

a. At quantities lower than approved by the FDA, Fabrazyme® was an off-label drug and was not fit, suitable, or safe for its intended purpose; and

b. In all other manners alleged herein.

328. Because Product Defendants affirmatively decided to sell patients, including Dr.

Schubert, Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide complete and accurate information to physicians and patients regarding the dose, its risks, and the duration with which such dose would be provided, and also duty to provide a drug which was safe and efficacious.

329. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, would be particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

330. Without limitation, Fabrazyme® was also defective because it lacked adequate label warnings and instructions for use associated ineffective dosing, thereby making the drug unreasonably dangerous in the dose it was supplied during the shortage, because:

a. There was a risk of harm that arose from the intended or reasonably foreseeable use of the Fabrazyme® in such off label quantities that was known to Product Defendants and/or that was scientifically discoverable at the time of the exposure;

b. There was a risk of harm that arose from the intended or reasonably foreseeable use of the Fabrazyme® for patients with advanced cardiac

or renal symptoms that was known to Product Defendants and/or that was scientifically discoverable at the time of the exposure;

c. Product Defendants, who exercised substantial control over the content of the warnings and/or instructions, knew or should have known of the risks at the time they marketed and sold the reduced doses, but failed to provide warnings of the dangers;

d. Product Defendants, who exercised substantial control over the content of the warnings and/or instructions, knew or should have known that warning patients and physicians, including Dr. Schubert and Dr. Longo, of the uncertainty of the projections for the length of the shortage was critical to patients and physicians in making correct treatment decisions; and

e. The above risks were not communicated to patient in product labeling and communications provided to patients at the time the reduced dose was offered, and;

f. In other manners alleged herein.

331. The absence of such warnings and/or instructions rendered the product unsafe for its intended or reasonably foreseeable use.

332. Such failure to warn and/or instruct was a legal and proximate cause of Dr. Schubert's death and the damages asserted herein.

333. Supplying Fabrazyme® in 0.3 mg/kg amounts and supplying the drug with a recommendation for "dose skipping" increased the harm to Dr. Schubert, because it caused Dr. Schubert to delay or forego seeking other treatment alternatives.

334. The Product Defendants were, at all times relevant, engaged in the business of designing, creating, manufacturing, testing, labeling, packaging, supplying, marketing, selling, advertising, warning, and otherwise distributing and placing in the stream of commerce the drug Fabrazyme®. The Product Defendants are strictly liable to Plaintiff as follows under the Food, Drug, and Cosmetics Act 21 U.S.C. §351(a-d) regarding adulterated products, 21 U.S.C. §352(f) regarding adequate warning and labeling, 21 U.S.C. §355(j) regarding the statutory approval process for testing of previously unapproved doses, and 21 U.S.C. §356a(a) regarding testing required for substantial manufacturing changes; as well as being strictly liable under the Bayh-Dole Act 35 U.S.C. §200 regarding the prohibition of unreasonable use or non-use of Bayh-Dole regulated inventions which are necessary for human health.

335. At all relevant times hereto, the Product Defendants' conduct as set forth herein was done with conscious indifference and knowing and reckless disregard for the safety and well-being of individuals, including Dr. Schubert, and such conduct justifies the imposition of punitive damages against the Product Defendants.

336. The aforementioned design, manufacture, and warning defects existed at the time or before Fabrazyme® was placed into the stream of commerce and caused Dr. Schubert and Plaintiff the harms alleged herein.

THIRD CLAIM FOR RELIEF
(Common law Breach of Warranty v. Product Defendants)

337. Plaintiff incorporates all preceding paragraphs and further alleges the following:

338. The Product Defendants made express and implied affirmations of fact or promises regarding the safety and effectiveness of the Fabrazyme®, including, but not limited to

(a) there was a clinical basis to believe that Fabrazyme® in 0.3 mg/kg doses would be efficacious for patients, (b) the duration for which Fabrazyme® would need to be purchased at a reduced dose would be limited to between 6 weeks and, at most, 2-3 months, (c) during the period of the supply interruption, a medically acceptable option for patients was to skip infusions and take Fabrazyme® in doses equivalent to 30% of the FDA-approved label dose, which became part of the basis of the bargain, and (d) in full dose, Fabrazyme® was clinically efficacious. The Product Defendants expressly and/or impliedly communicated that the reduced-dose Fabrazyme® conforms to their affirmations.

339. As described herein, Fabrazyme® failed to conform to the Product Defendants' express warranties because in fact it was not be therapeutic to Dr. Schubert and many other patients and patients had to purchase Fabrazyme® at reduced doses for significantly longer before they could purchase Fabrazyme® at full doses.

340. Product Defendants therefore breached their common law warranties.

341. As a direct and proximate cause of the breach of these warranties as set forth in paragraphs 1–308 and 337–340, Dr. Schubert and Plaintiff suffered damages as set forth herein.

FOURTH CLAIM FOR RELIEF
(Breach of Implied Warranty of Fitness v. Product Defendants–Uniform Commercial Code 70A-2-315)

342. Plaintiff incorporates all preceding paragraphs and further alleges the following:

343. Product Defendants had reason to know that the particular purpose for which the drug Fabrazyme® was to be used.

344. By selling Fabrazyme®, first in 1.0 mg/kg label doses and later in 0.3 mg/kg "off label" doses, Product Defendants implicitly warranted that Fabrazyme® was fit for the purpose for which it was used by Dr. Schubert.

345. The purpose of Fabrazyme®, as marketed by Product Defendants, is to treat Fabry patients, and specifically to prevent or slow progression of conditions which develop in Fabry patients, including but not limited to progressive cardiac disease and renal failure.

346. In October, 2009 Product Defendants knew, and advised the Australian medical authority as follows: "the suggestion that the dose of Fabrazyme® could be reduced to 0.2 mg/kg with little or no loss of efficacy is inappropriate and unsupported by the current body of evidence. Applying such [a reduction] across the board will have significant clinical consequences for patients, with the expectation that many would suffer irreversible harm and a result of insufficient dosing."

347. Despite such knowledge, Product Defendants marketed and sold Fabrazyme® to Dr. Schubert and others with clinically significant cardiac disease in similar, reduced, "off label" doses and continued to do so even after Dr. Schubert's cardiac symptoms became severe.

348. Additionally, based on recent testimony from Product Defendants' senior medical officer, Fabrazyme® appears to be unsuitable for the medical purpose for which it is marketed, even in its full dose. Dr. Gruskin, who is Product Defendants' Vice President for U.S. Medical Affairs, has testified that despite nearly 10 years experience with this drug, Product Defendants currently lack medical evidence to show that Fabrazyme®, even at full dose, changes clinical outcome for Fabry patients. *See* fn. 3, supra.

349. Dr. Gruskin has also testified that "when there is fibrosis in the heart on MRI there is either minimal or no impact of Fabrazyme." Deposition of Daniel Gruskin 65:10-12.

350. If Dr. Gruskin's medical assessment is correct, Fabrazyme® is not fit for the purpose for which it is sold to patients, including but not limited to patients with significant cardiac disease, as it may provide no clinical benefit.

351. Nevertheless, Product Defendants sold Fabrazyme® to Dr. Schubert and many other patients in both full and reduced doses without disclosing any of the above limitations as to efficacy.

352. Product Defendants had reason to know that Dr. Schubert and Plaintiff were relying on the skill and judgment of Product Defendants to select or furnish suitable products.

353. The Fabrazyme® doses and/or quality supplied by Product Defendants were unfit for the particular purposes for the reasons set forth in this complaint, including paragraphs1–308 and 342–352.

354. Thus, Product Defendants breached the implied warranty of fitness for a particular purpose, and this breach of warranty was the proximate cause of Dr. Schubert's death and Plaintiff's injuries.

355. As such, Plaintiff is entitled to recover under Utah Code Ann. § 70A-2-315.

FIFTH CLAIM FOR RELIEF
(Breach of Implied Warranty of Merchantability v. Product Defendants)

356. Plaintiff incorporates all preceding paragraphs and further alleges the following:

357. Product Defendants are merchants with respect to Fabrazyme® and implicitly warranted that it was merchantable.

358. However, the Fabrazyme® was not merchantable because it contained the defects outlined in paragraphs 1–308 and 342–352.

359. Thus, Product Defendants breached the implied warranty of merchantability, and this breach of warranty was the proximate cause of Dr. Schubert's death and Plaintiff's injuries.

360. Plaintiff is entitled to recover under Utah Code Ann. § 70A-2-314.

SIXTH CLAIM FOR RELIEF
(Negligence v. the University of Utah)

361. Plaintiff incorporates all preceding paragraphs and further alleges the following:

362. As his treating physician, Dr. Longo owed a duty of care to Dr. Schubert.

363. Dr. Longo was negligent and breached the standard of care by his acts or omissions detailed in paragraphs 1–308, including, but not limited to:

a. By recommending that Dr. Schubert skip doses between July and September, 2009, when Dr. Schubert was not an appropriate candidate to do so;

b. By failing to warn Dr. Schubert of the risks of dose skipping and taking reduced dose of Fabrazyme;

c. By agreeing to assist Dr. Schubert in obtaining Replagal® and thereafter failing to apply for an emergency compassionate use exemption from the FDA based upon a showing of urgent medical need;

d. By failing to advise Dr. Schubert, at the commencement of the shortage, that another drug (Replagal) was an acceptable alternative treatment for Fabry Disease, that Replagal® had been approved by the Canadian, European, Mexican and other health agencies, and that Replagal® was not subject to supply shortages;

e. By failing to maintain current as to how to rapidly access Replagal® and/or other therapies to treat Fabry Disease once the shortage of Fabrazyme® was announced; and

f. Such other acts of negligence as may be shown by the proof at trial.

364. As a direct and proximate result of Dr. Longo's negligence and breach of the standard of care, Dr. Schubert died.

365. Plaintiff, on behalf of Decedent's estate and heirs, sustained damages as a result of Dr. Longo's acts and/or omissions as described herein, including:

a. Economic damages including, without limitation, funeral and burial expenses, future wage loss, loss of economic support, and other economic damages as the evidence may show at trial, and

b. Non-economic damages including, without limitation, pain and suffering, mental anguish, loss of consortium, companionship, society, love of a husband and father, and any other non-economic damages as the evidence may show at trial.

SEVENTH CLAIM FOR RELIEF
(*Respondeat Superior* v. the University of Utah)

366. Plaintiff incorporates all preceding paragraphs and further alleges the following:

367. During all relevant times, Dr. Longo was an employee, agent, or an individual acting under the direction and control of the University of Utah.

368. During all relevant times, Dr. Longo acted within the course and scope of his employment at the University of Utah as the treating physician for Dr. Schubert.

369. As previously described, Dr. Longo was negligent in his care of Dr. Schubert and his actions were a direct and proximate cause of the damages sustained by Dr. Schubert and Plaintiff.

370. The University of Utah is vicariously liable for the conduct of Dr. Longo, under the doctrine of *respondeat superior*, and is therefore liable for the damages sustained by Dr. Schubert and Plaintiff.

EIGHT CLAIM FOR RELIEF
(*Alternatively*, Apparent Agency v. the University of Utah)

371. Plaintiff incorporates all preceding paragraphs and further alleges the following:

372. During all relevant times, the University of Utah clothed Dr. Longo as its employee.

373. Dr. Longo was, *inter alia*, listed on the University of Utah website and in the employee directory as an employee-physician in the Pediatrics department.

374. Plaintiff and Dr. Schubert reasonably relied on this when they decided that Dr. Schubert will treat with Dr. Longo.

375. As previously described, Dr. Longo was negligent in his care of Decedent and his actions were a direct and proximate cause of the damages sustained by Plaintiff.

376. The University of Utah is liable for Dr. Longo's negligence under the doctrine of apparent agency and is therefore liable for the damages sustained by Plaintiff.

NINTH CLAIM FOR RELIEF
(Breach of Fiduciary Duty v. the University of Utah)

377. Plaintiff incorporates all preceding paragraphs and further alleges the following:

378. Dr. Longo owed Dr. Schubert a fiduciary duty when treating him, and specifically, advising him about his medication and treatment options available to him.

379. Dr. Longo breached his fiduciary duty by not disclosing to Dr. Schubert that he had a financial relationship with Product Defendants, by failing to alter Dr. Schubert's medication after the Fabrazyme® shortage began, and by applying for a "treatment" compassionate use exemption instead of an "emergency" compassionate use exemption.

380. Upon information and belief, other drug alternatives were available to Dr. Longo to choose from during all relevant times.

381. As a direct and proximate result of Dr. Longo's breach of his fiduciary duty, Dr. Schubert died.

382. Plaintiff, on behalf of Decedent's estate and heirs, sustained damages as a result of Dr. Longo's acts and/or omissions as described herein, including:

a. Economic damages including, without limitation, funeral and burial expenses, future wage loss, loss of economic support, and other economic damages as the evidence may show at trial, and

b. Non-economic damages including, without limitation, pain and suffering, mental anguish, loss of consortium, companionship, society, love of a husband and father, and any other non-economic damages as the evidence may show at trial.

TENTH CLAIM FOR RELIEF
(Negligent Misrepresentation under RST § 311 v. Product Defendants)

383. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

384. Product Defendants sold Fabrazyme® and had a pecuniary interest in selling Fabrazyme®.

385. Because Product Defendants affirmatively decided to sell to patients, including Dr. Schubert, Fabrazyme® at doses lower than were approved by the FDA, including doses during the period where Product Defendants caused doses to be skipped in August and September and at all times thereafter where Product Defendants sold Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide truthful, accurate, and non-misleading information to physicians and patients regarding the doses of Fabrazyme®, medical risks of skipping infusions and later taking a reduced dose of Fabrazyme®, the likely duration of the shortage, the treatment alternatives available during the shortage, the meaning of the relevant medical studies in regard to the risks assumed by "off label" treatment with Fabrazyme®, and other facts relevant to Dr. Schubert's ERT therapy, as detailed in paragraphs 1–308 and 342–352.

386. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

387. As detailed above in paragraphs 1–308 of this Complaint, Product Defendants negligently, recklessly, and/or intentionally affirmatively gave false information to Dr. Schubert and Plaintiff. Product Defendants either knew or could have known such information was incorrect had Product Defendants acted reasonably. The misrepresentations, made to Dr. Longo, Dr. Shubert, and/or Mrs. Shubert caused Dr. Schubert to agree to skip doses of Fabrazyme®, to later take the reduced dose offered him, and to forego other treatment options that Dr. Schubert could have more urgently sought. The misrepresentations include, but are not limited to, all affirmative communications detailed in Exhibit A to this Complaint.

388. Dr. Longo, Dr. Schubert, and Plaintiff relied on such information, which was false, and which reliance was a direct and proximate resulted in the physical harms detailed herein.

389. Product Defendants are thus liable for the physical harm caused to Dr. Schubert.

390. As such, Plaintiff is entitled to relief.

ELEVENTH CLAIM FOR RELIEF
(Negligent Misrepresentation under RST § 551 v. Product Defendants)

391. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

392. Product Defendants sold Fabrazyme® and had a pecuniary interest in selling Fabrazyme®.

393. Because Product Defendants affirmatively decided to sell to patients, including Dr. Schubert, Fabrazyme® at doses lower than were approved by the FDA, including doses during the period where Product Defendants caused doses to be skipped in August and September, 2009 and at all times thereafter where Product Defendants sold Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide

truthful, accurate, and non-misleading information to physicians and patients regarding the dose, medical risks of skipping infusions and later taking reduced dose of Fabrazyme®, the likely duration of the shortage, the treatment alternatives available during the shortage, the meaning of the relevant medical studies in regard to the risks assumed by "off label" treatment with Fabrazyme®, and other relevant facts as detailed in paragraphs 1–308 and 342–352.

394. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

395. As detailed above in paragraphs 1–308 of this Complaint, Product Defendants negligently, recklessly, and/or intentionally affirmatively gave false information to Dr. Schubert and Plaintiff. Product Defendants either knew or would have known such information was incorrect had Product Defendants acted reasonably. The misrepresentations, made to Dr. Longo, Dr. Shubert, and Mrs. Shubert caused Dr. Schubert to agree to skip doses of Fabrazyme® and later take the reduced dose offered him, and thereby continue to purchase Fabrazyme®, and to forego other treatment options that Dr. Schubert could have more urgently sought. The

misrepresentations include, but are not limited to, all affirmative communications detailed in Exhibit A to this Complaint.

396. Product Defendants alleged that Fabrazyme® was effective at doses lower than the FDA-approved 1.0 mg/kg dose without scientifically significant knowledge to support their claims. Product Defendants did so through many actions including, but not limited to, supporting the use of Fabrazyme® at doses lower than was approved by the FDA and by referring patients and physicians to only the Lubanda study, knowing that the Lubanda study could be improperly interpreted as clinical support for a dose of 0.3 mg/kg Fabrazyme®.

397. Product Defendants informed, inter alia, Dr. Schubert that it projected the shortage to be of a limited duration when it knew, or with reasonable investigation should have known, that the shortage would last much longer.

398. As acknowledged by Dr. Gruskin in various emails, some of which are referenced in this Complaint, Product Defendants communicated information regarding the length of the shortage with knowing and reckless disregard for the truth.

399. As detailed in paragraphs 1–308, Product Defendants made these false representations with knowledge that they were false and/or recklessly, knowing that they had insufficient knowledge on which to base such representation.

400. Product Defendants made such representations to induce Dr. Schubert to continue to purchase Fabrazyme®.

401. But for these false representations, Plaintiff and Dr. Schubert would have been more aggressive in obtaining alternative treatments. Instead, Plaintiff and Dr. Schubert relied on those representations and believed, inter alia, that Fabrazyme® would be effective in the reduced

dose and that the shortage would be only temporary and confined to a few months at most and thus continued to purchase Fabrazyme®. Plaintiff and Dr. Schubert did so to their detriment.

402. Plaintiff is entitled compensatory and punitive damages as fully set forth herein.

TWELFTH CLAIM FOR RELIEF
(Strict Liability Misrepresentation under RST § 402B v. Product Defendants)

403. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

404. Product Defendants sold Fabrazyme® and had a pecuniary interest in selling Fabrazyme®.

405. As described in paragraphs 1–308 of this Complaint, Product Defendants made materially false representations to the general public and to Dr. Schubert, Mrs. Schubert, and Dr. Longo about the effectiveness of Fabrazyme® at doses lower than the FDA-approved 1.0 mg/kg dose administered every two weeks and the duration of the Fabrazyme® supply shortage. These false representations include, but are not limited to, those specifically detailed in Exhibit A to this Complaint.

406. But for these false representations, Plaintiff and Dr. Schubert would not have agreed to first skip and then take a reduced dose and would have been more aggressive in obtaining alternative treatments. Instead, Plaintiff and Dr. Schubert relied on those representations and believed, inter alia, that Fabrazyme® would be effective in the reduced dose and that the shortage would be only temporary and confined to a few months at most. Plaintiff and Dr. Schubert did so to their detriment.

407. Plaintiff is entitled to relief under Utah common law.

THIRTEENTH CLAIM FOR RELIEF
(Fraudulent Misrepresentation v. Product Defendants)

408. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

409. Product Defendants sold Fabrazyme® and had a pecuniary interest in selling Fabrazyme®.

410. Because Product Defendants affirmatively decided to sell to patients, including Dr. Schubert, Fabrazyme® at doses lower than were approved by the FDA, including doses during the period where Product Defendants caused doses to be skipped in August and September, 2009 and at all times thereafter where Product Defendants sold Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide truthful, accurate, and non-misleading information to physicians and patients regarding the doses of Fabrazyme®, medical risks of skipping infusions and later taking reduced doses of Fabrazyme®, the likely duration of the shortage, the treatment alternatives available during the shortage, the meaning of the relevant medical studies in regard to the risks assumed by "off label" treatment with Fabrazyme®, and other relevant facts as detailed in paragraphs 1–308 and 342–352.

411. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor

clinically relevant on this issue.

412. As detailed above in paragraphs 1–308 and this Complaint, Product Defendants negligently, recklessly while knowingly they lacked sufficient knowledge upon which to base such a representation, and/or intentionally affirmatively gave false information to Dr. Schubert and Plaintiff. Product Defendants either knew or could have known such information was incorrect had Product Defendants acted reasonably. The misrepresentations, made to Dr. Longo, Dr. Shubert, and Mrs. Shubert caused Dr. Schubert to agree to skip doses of Fabrazyme® and later take the reduced dose offered him, and thereby continue to purchase Fabrazyme®, and to forego other treatment options that Dr. Schubert could have more urgently sought. The misrepresentations include, but are not limited to, all affirmative communications detailed in Exhibit A to this Complaint.

413. Product Defendants alleged that Fabrazyme® was effective at doses lower than the FDA-approved 1.0 mg/kg dose without scientifically significant knowledge to support their claims. Product Defendants did so through many actions including, but not limited to, supporting the use of Fabrazyme® at doses lower than was approved by the FDA and by referring patients and physicians to only the Lubanda study, knowing that the Lubanda study could be improperly interpreted as clinical support for a dose of 0.3 mg/kg Fabrazyme®.

414. Product Defendants informed, inter alia, Dr. Schubert that it projected the shortage to be significantly shorter than it actually was when Product Defendants knew that the shortage would last much longer, or at least knew they lacked sufficient knowledge to state otherwise. Product Defendants did so with knowing and reckless disregard for the truth.

415. As detailed in paragraphs 1–308, Product Defendants made these false representations with knowledge that they were false and/or recklessly, knowing that they had insufficient knowledge on which to base such representation.

416. But for these false representations, Plaintiff and Dr. Schubert would not have agreed to skip doses in August and September, 2009 and would have been more aggressive in obtaining alternative treatments. Instead, Plaintiff and Dr. Schubert relied on those representations and believed, inter alia, that Fabrazyme® would be effective in the reduced dose and that the shortage would be only temporary and confined to a few months at most. Plaintiff and Dr. Schubert did so to their detriment.

417. Additionally, as described in paragraphs 1–308, Product Defendants knew their projections related to the length of the shortage were based on false projections or were made recklessly with knowledge that they lacked sufficient information upon which to base such representations.

418. As described in paragraphs 1–308, Product Defendants knew, or should have known and avoided such knowledge with reckless disregard for the truth, or at least knew they lacked sufficient information to assert otherwise, that Fabrazyme® would not be effective for many patients at such a reduced dose, especially patients with advanced Fabry Disease like Dr. Schubert, that patients, like Dr. Schubert, who exhibited clinical decline on the reduced dose would suffer irreversible harm if not immediately returned to a full dose of Fabrazyme®.

419. However, in addition to the actions described in the preceding paragraphs, Product Defendants breached their duty when:

a. Dr. Gruskin was supplied with knowingly false information to convey to the US FSWG in June and September, 2009, about the length of the shortage;

b. Dr. Gruskin was supplied with knowingly false information to convey to the US FSWG in June and September, 2009, that the guidelines to be developed by the US FSWG were to be adopted in all regions of the world;

c. Dr. Gruskin affirmatively conveyed this information to the US FSWG, as more fully described above, in June and September 2009;

d. Dr. Gruskin and other officials of Product Defendants prepared and distributed letters, with Dr. Gruskin's and/or other employee's signatures, to patients and physicians on or about July 2, 2009, September 24, 2009, December 2, 2009, and February 17, 2010, explaining the length of the shortage, which repeatedly was found to be false, and the US FSWG's endorsement of Product Defendants' plan, as more fully described above;

e. Product Defendants supplied false information to the FDA in its responses to the FDA letter dated July 6, 2009, which response was dated July 9, 2009 and signed by Alex Kuta, concerning Product Defendants' ability to assure adequate supplies and to provide a geographically equitable allocation of Fabrazyme;

f. In town hall communications wherein various Product Defendants employees presented information and responded to questions while patients and

physicians could call in and listen to learn more about the shortage, as detailed Exhibit A;

g. In giving forms with frequently asked questions and Product Defendants' prepared responses that provided false information regarding the length of the shortage and the efficacy of Fabrazyme® at doses lower than the FDA-approved dose, as detailed in this Complaint; and

h. Other acts as plead, or which may be discovered during this case.

420. Product Defendants used the US FSWG as a vehicle whereby to perpetrate fraudulent misrepresentations on Dr. Schubert, Plaintiff, Dr. Longo, and other patients and physicians.

421. Product Defendants knowingly or recklessly, while knowing they lacked sufficient knowledge upon which to base such a representation, made such fraudulent misrepresentations with the goal of inducing Dr. Schubert, Plaintiff, and Dr. Longo to continue to use Fabrazyme® on the reduced dose during the shortage.

422. Dr. Schubert, Plaintiff, and Dr. Longo reasonably relied on Product Defendants fraudulent misrepresentations, as set forth in paragraphs 134, 135, 143, 156, 196, 224, 225, and 234 of this Complaint, in Exhibit A to this Complaint, and elsewhere in this Complaint, which reliance was a direct and proximate cause of the harms alleged herein.

423. Plaintiff is entitled to compensatory and punitive damages as set forth herein.

FOURTEENTH CLAIM FOR RELIEF
(Fraudulent Concealment v. Product Defendants)

424. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

425. Product Defendants manufactured and distributed Fabrazyme®, a prescription drug, under market protection pursuant to the Orphan Drug Act that restricted access to the market for Fabry patients and gave Product Defendants a monopoly of Fabry ERT therapy in the Unities States.

426. Product Defendants thus owed a duty to inform physicians and patients, including Dr. Longo and Dr. Schubert, of any and all information related to Fabrazyme® where, without such knowledge, harm could be suffered by the end user of Fabrazyme®.

427. Additionally, because Product Defendants continued to manufacture and distributed Fabrazyme® at doses lower than was approved by the FDA, Product Defendants owed a duty to inform those they communicated with, including Dr. Longo and Dr. Schubert, of all information necessary to make informed treatment decisions.

428. As detailed in paragraphs 1–308, such information includes, but is not limited to: the lack of efficacy of treatment with Fabrazyme; the lack of efficacy of treatment with Fabrazyme® at doses Product Defendants required, recommended, encouraged, or otherwise recklessly caused to be administered, where such doses were significantly lower or different than the FDA-approved dose; the increasing lack of efficacy of Fabrazyme® at reduced doses over increased periods of time; the fact that patients who remained stable while they received Fabrazyme® at a full dose could deteriorate on lower doses to a point where recovery was impossible; and all facts related to the length of time Product Defendants required, or recklessly caused, patients to be administered doses lower than the dose approved by the FDA.

429. The facts in the preceding paragraph were material to decisions regarding treatment with Fabrazyme® in that, had they been properly communicated, physicians and

patients using Fabrazyme®, including Dr. Longo and Dr. Schubert, would have been more active and aggressive in seeking alternative treatments, including, but not limited to, accessing Replagal®.

430. Because Product Defendants affirmatively decided to sell patients, including Dr. Schubert, Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide reasonably complete, truthful, accurate and non-misleading information to physicians and patients regarding the dose, its risks, and the duration with which such dose would be provided, and also a duty to provide a drug which was safe and efficacious.

431. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

432. As detailed in paragraphs 1–308, Product Defendants fraudulently concealed their knowledge, as shown by their response to the Australian government's effort to approve Fabrazyme® for use at 0.2 mg/kg, that reducing the dose of Fabrazyme® to significantly lower than the FDA-approved dose of 1.0 mg/kg every-other-week "across the board," as Product Defendants did in the U.S., "would have significant clinical consequences for patients, **with the**

expectation that many would suffer irreversible harm as a result of insufficient dosing," that "**treatment at a higher dose is necessary and may be life-saving**," that the suggestion to reduce the dose of Fabrazyme® to 0.2 mg/kg in all patients "ignores the cumulative evidence in the extant literature," that such a reduction could occur "with little or no loss of efficacy is conjectural," that the medical conclusion from the Vedder study was that a dose of 0.2 mg/kg of Fabrazyme® would not "elicit[] a clinically relevant response to treatment in many Fabry patients," and that a "**blanket dose adjustment would be insane**." GENZYME013854; GENZYME013847; GENZYME013840.

433. As detailed in paragraphs 1–308, Product Defendants intentionally fraudulently concealed such information from physicians and patients, including Dr. Schubert and Dr. Longo, by not communicating it to the US FSWG or to patients and physicians.

434. Additionally, Product Defendants fraudulently concealed Product Defendants' knowledge that, as stated in the deposition of Dr. Gruskin, Product Defendants' Vice President of U.S. Medical Affairs, that Fabrazyme® has not been proven to change clinical outcomes of patients, including, but not limited to, those with advanced stage disease.

435. Product Defendants did not inform Plaintiff, Dr. Schubert, or Dr. Longo of these material facts, including those listed above.

436. Because Plaintiff, Dr. Schubert, and Dr. Longo did not receive such facts, Dr. Schubert skipped doses of Fabrazyme® in August and September, 2009, Dr. Schubert continued to purchase Fabrazyme® at a dose 70% lower than was approved by the FDA thereafter, and they did not actively pursue alternative treatments in a timely manner.

437. Thus, as a direct and proximate result of the Product Defendant's fraudulent concealment of these material facts, Dr. Schubert and Plaintiff suffered the harms alleged herein.

438. Plaintiff is entitled to relief as fully set forth in this Complaint.

FIFTEENTH CLAIM FOR RELIEF
(Fraudulent Inducement v. Product Defendants)

439. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

440. Product Defendants sold Fabrazyme® and had a pecuniary interest in selling Fabrazyme®.

441. Product Defendants sold multiple doses of Fabrazyme® to Dr. Schubert, thereby creating multiple contracts for the sale of Fabrazyme®.

442. Each of Dr. Schubert's purchases of doses of Fabrazyme® created a contract whereby Dr. Schubert was purchasing a treatment for Fabry Disease which was, as represented by Product Defendants, supposed to improve and/or maintain Dr. Schubert's clinical status.

443. Because Product Defendants affirmatively decided to sell patients, including Dr. Schubert, Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide complete and accurate information to physicians and patients regarding the dose, its risks, and the duration with which such reduced dose would be provided, and also duty to provide a drug which was safe and efficacious.

444. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of

medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

445. Product Defendants had knowledge of facts that were material to Dr. Schubert's decision on whether to purchase Fabrazyme® and that were unknown and undiscoverable to Dr. Schubert, Mrs. Schubert, and Dr. Longo. Such material facts included, but were not limited to, that, as alleged in paragraphs 1–308, Product Defendants knew that Fabrazyme® was not effective at doses lower than the FDA-approved dose and that many patients would suffer irreversible harm on such reduced doses, that Product Defendants knew that Fabrazyme® was not beneficial to patients with advanced Fabry Disease, and that Product Defendants knew the shortage of Fabrazyme® would likely be much longer than was represented to Dr. Schubert, Mrs. Schubert, and Dr. Longo.

446. Despite such knowledge, Product Defendants made multiple affirmative misrepresentations of fact, as detailed in paragraphs 1–308 of this Complaint and more specifically in Exhibit A to this Complaint.

447. As described in paragraphs 1–308 and Exhibit A, Product Defendants induced Dr. Schubert to continue to receive, and Dr. Longo to continue to prescribe, Fabrazyme® in non-approved doses and dose schedules by affirmatively and repeatedly misrepresenting the length of the shortage of Fabrazyme® and by misrepresenting that the problems with the Allston plant and bioreactors had been resolved through its letters and attached documents, drafted by a committee of Product Defendants officials and signed by Dr. Gruskin and other Product Defendants

employees, and sent to patients and physicians, including Dr. Schubert and Dr. Longo, on or about June 16, 2009, July 1, 2009, July 2, 2009, September 23, 2009, December 1, 2009, and February 17, 2010, and through other communications as detailed herein and in Exhibit A.

448. Every one of such representations was false and/or misleading.

449. As described in paragraphs 1–308, Product Defendants knew these representations were false and/or misleading, or in the alternative, were false and made recklessly with knowledge that they lacked sufficient information upon which to make such representations, knowing they could not give reasonably accurate information that the duration of the supply shortage would be very brief, knowing their failures to remediate the deficiencies of the Allston plant were likely to cause future disruptions, and other reasons which are detailed herein.

450. Additionally, and as described in paragraphs 1–308 and Exhibit A, Product Defendants induced Dr. Schubert to continue to receive, and Dr. Longo to continue to prescribe, Fabrazyme® by affirmatively and repeatedly misleading Dr. Longo and Dr. Schubert into believing Fabrazyme® would be reasonably efficacious while skipping doses in August and September, 2009, and would be reasonably efficacious in doses 70% less than what was approved by the FDA thereafter, through its letters and attached documents, drafted by a committee of Product Defendants officials and signed by Dr. Gruskin, and sent to patients and physicians, including Dr. Schubert and Dr. Longo, on or about June 16, 2009, July 1, 2009, July 6, 2009, September 23, 2009, December 1, 2009, and February 17, 2010, and through other means detailed herein and in Exhibit A; by continuing to sell Fabrazyme® at reduced doses with knowledge of Dr. Schubert's clinical condition and the actual likely length of the shortage; and

by referring in such communications to the Lubanda study as a means of portraying clinical support for Fabrazyme® at a dose of 0.3 mg/kg.

451. Additionally, Product Defendants represented that Fabrazyme® was proper to treat Dr. Schubert by selling Fabrazyme® to Dr. Schubert with knowledge of Dr. Schubert's health condition, by selling Fabrazyme® at doses lower than the FDA-approved dose to Dr. Schubert with knowledge of Dr. Schubert's health condition, and by selling Fabrazyme® at doses lower than approved by the FDA with knowledge that the shortage would be significantly longer than was represented to Dr. Schubert and Dr. Longo.

452. As described in paragraphs 1–308, at or about the very same time Product Defendants were representing to Dr. Schubert and Dr. Longo that it would be reasonably safe for patients to take reduced doses or doses on an interrupted schedule, Product Defendants were actively warning the Australian medical authority of significant risks associates with similarly reduced doses.

453. Thus, Product Defendants made its statements to United States Fabry patients, including Dr. Schubert, with knowing and reckless disregard for their untruthfulness and for the safety of consumers of Fabrazyme®, or, in the alternative, recklessly while knowing they lacked sufficient knowledge upon which to base such representations.

454. As described in paragraphs 1–308, all such representations were false and were made knowingly, with reckless disregard for the truth, and/or recklessly while knowing they lacked sufficient knowledge upon which to base such representations.

455. As described in paragraphs 1–308, Product Defendants actively concealed from Dr. Schubert and Dr. Longo material facts as to the effectiveness of Fabrazyme® at doses lower

than the FDA-approved dose and the true, likely length of the shortage in an effort to maintain Dr. Schubert as a Fabrazyme® client and to induce Dr. Schubert to continue to purchase Fabrazyme®.

456. As detailed in paragraphs 1–308 and Exhibit A, Product Defendants knowingly and/or recklessly, knowing they lacked sufficient knowledge upon which to base such representations, made the above-described misrepresentations which were relied upon by Dr. Schubert and Dr. Longo in their making decisions to continue to purchase and use Fabrazyme® as a treatment for Dr. Schubert's Fabry Disease.

457. As detailed in paragraphs 1–308 and Exhibit A, Product Defendants made these misrepresentations with the intent of inducing Dr. Schubert to continue to receive, and Dr. Longo to continue to prescribe, Fabrazyme® as a treatment for Dr. Schubert's Fabry Disease.

458. As described in paragraphs 1–308 and Exhibit A, these statements were false and/or misleading, and resulted in Dr. Schubert's accelerated medical decline and premature death.

459. In the alternative, as described in paragraphs 1–308 and 342–352, Product Defendants affirmatively misled Dr. Schubert and Plaintiff to believe that Fabrazyme® was efficacious at a full FDA-approved dose. Product Defendants made material and false representations to Dr. Longo about the efficacy of Fabrazyme® which were relayed to Dr. Schubert, as described in paragraphs 1–308 and Exhibit A.

460. That Fabrazyme® is not proven to improve clinical outcomes is a fact that was known to Product Defendants and could not reasonably have been known by Dr. Schubert.

461. As described herein, Product Defendants, as evidenced through the testimony of Daniel Gruskin, their Vice President of U.S. Medical Affairs, knew that Fabrazyme® had, and still has, not been proven to improve clinical outcomes of patients.

462. As described in paragraphs 1–308 and Exhibit A, Product Defendants made, through its employees, these representations to induce Dr. Longo to prescribe, and Dr. Schubert to receive, Fabrazyme® to treat Dr. Schubert's Fabry Disease.

463. As described in paragraphs 1–308 and Exhibit A, Dr. Schubert and Dr. Longo relied on these above misrepresentations, and all affirmative misrepresentations detailed in this Complaint and Exhibit A hereto, which were material, in deciding to purchase and use Fabrazyme® to treat Dr. Schubert's Fabry Disease.

464. As a result of Dr. Schubert and Dr. Longo's reliance on Product Defendants' fraudulent representations and concealment of material facts, Dr. Schubert suffered the physical and emotional harms alleged herein and Dr. Schubert and Plaintiff suffered financial harm by purchasing, directly and through insurance, Fabrazyme®.

465. Dr. Schubert and Plaintiff paid significant insurance premiums and deductibles which would not have been necessary had Dr. Schubert received alternative, less expensive treatments.

466. Dr. Schubert and Plaintiff's insurer during the shortage, Blue Shield of Idaho, has noticed Plaintiff of its subrogation claim to recover for their payments for Fabrazyme® and Fabrazyme® treatment which are awarded Plaintiff as a result of this suit.

467. Thus, Plaintiff is entitled to damages.

SIXTEENTH CLAIM FOR RELIEF

(Fraudulent Nondisclosure and Constructive Fraud based on a "Confidential Relationship" v. Product Defendants)

468. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

469. As shown in paragraphs 1–308 and Exhibit A, from June 2009 through March 2010 , Product Defendants stood in a confidential relationship with Dr. Schubert, Plaintiff, and Dr. Longo for the following reasons, among others:

a. Product Defendants manufactured a life-saving drug under a license that gave it an effective monopoly on ERT treatment for Fabry Disease with U.S. patients;

b. Product Defendants were able to exercise extraordinary influence over Plaintiff, Dr. Schubert, and Dr. Longo because Product Defendants controlled sole access to information that was critical to Dr. Schubert's critical medical care decisions during the shortage, particularly information about (i) how long the Fabrazyme® supply interruption and shortage would last, (ii) whether there were medical risks associated with skipping doses or taking reduced doses of Fabrazyme® during the supply interruption; and (iii) the efficacy of Fabrazyme® in reduced doses or altered dosing schedules;

c. Product Defendants acted in a way to influence Dr. Schubert's treatment decisions during the shortage by supplying information and communications to the FDA, US FSWG, physicians, including Dr. Longo, and patients, including Dr. Schubert;

d. Product Defendants knew or should have known that Dr. Shubert and his treating physician, Dr. Longo, reposed trust and confidence in Product

Defendants and reasonably relied upon Product Defendants' information and guidance to make critical medical decisions. In fact, as described herein, Product Defendants set about to shape decisions of the US FSWG in order to ensure that its own treatment and allocation recommendations would be followed after the supply interruption was announced;

e. Product Defendants had been supplying Fabrazyme® to Dr. Schubert as a treatment for his Fabry Disease for several years before the shortage; and

f. For other reasons described herein.

470. By virtue of its confidential relationship with Dr. Schubert, Plaintiff, and Dr. Longo, Product Defendants owed Dr. Schubert and Plaintiff the duty to disclose material facts about Fabrazyme® as set forth in the body of this Complaint, which were known to Product Defendants, including, but not limited to: the supply interruption and shortage would or could last significantly longer than the time frames represented by Product Defendants during the period of June, 2009 to February, 2010; the problems at the Allston plant which affected production and supply had not been fully remedied during 2009; the reduced dose would not be efficacious for many patients; that Product Defendants were actively advocating against similarly reduced doses, showing Product Defendant's internal belief and knowledge that many, especially those like Dr. Schubert with advanced Fabry Disease, would suffer irreversible harm as a result of insufficient dosing, that treatment at a full dose is necessary and life-saving, and that reducing doses across all patients, without regard to clinical status, ignores the cumulative evidence in the extant literature; that patients with clinically significant cardiac disease were not suitable to skip

or take reduced doses; that once a patient started to decline on a reduced dose, restoring full dose would not prevent further medical deterioration in many Fabry patients; that alternate therapy, including Replagal® could be obtained during the shortage; that supplies of Fabrazyme® would likely be unreliable until as late as 2011 or 2012; and other statements that were material to Dr. Schubert's continued use and treatment with Fabrazyme®.

471. As described herein in paragraphs 1–308 and Exhibit A, Product Defendants intentionally and/or recklessly with intentional indifference to the truth and health of patients, failed to disclose the facts described in the preceding paragraph to Dr. Schubert, Plaintiff, and Dr. Longo.

472. Because Dr. Schubert, Plaintiff, and Dr. Longo did not receive truthful information on the above-mentioned subjects, which information was known to Product Defendants, Dr. Schubert, Plaintiff, and Dr. Longo followed Product Defendants' plan to have Dr. Schubert skip doses in August and September, 2009 and receive a dose 70% lower than was approved by the FDA thereafter, and Dr. Schubert, Plaintiff, and Dr. Longo delayed in seeking treatment alternatives. Such decisions and actions caused Dr. Schubert and Plaintiff the harms alleged herein.

473. Plaintiff has sustained damages as set forth in this complaint.

SEVENTEETH CLAIM FOR RELIEF
(Negligent Infliction of Emotional Distress v. Product Defendants)

474. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

475. Product Defendants promote Fabrazyme® as a life-preserving drug.

476. As described herein, Dr. Schubert and Plaintiff relied on Product Defendants' representations that Fabrazyme® was a life-preserving drug.

477. Because Product Defendants affirmatively decided to sell patients, including Dr. Schubert, Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide complete and accurate information to physicians and patients regarding the dose, its risks, and the duration with which such dose would be provided, and also duty to provide a drug which was safe and efficacious.

478. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

479. Product Defendants knowingly or negligently with reckless disregard for the truth caused Dr. Schubert and Plaintiff severe emotional distress by:

a. Negligently and/or recklessly causing a Fabrazyme® shortage;

b. Repeatedly misrepresenting to Dr. Schubert and Plaintiff the length of the shortage, causing Dr. Schubert and Mrs. Schubert to plan medical decisions around a resupply of full dose Fabrazyme® which did not occur;

c. Misrepresenting to Dr. Schubert that if he and other patients skipped doses of Fabrazyme® in August and September, 2009, a further reduction in dose could be avoided;

d. Misrepresenting the efficacy of Fabrazyme® at the FDA-approved dose;

e. Misrepresenting the efficacy of Fabrazyme® at doses lower than the FDA-approved dose;

f. Failing to timely respond to emergency requests for Fabrazyme® after Dr. Schubert's health began to deteriorate on reduced doses;

g. Failing to timely ship and supply Fabrazyme® to Dr. Schubert in February, 2010 after additional doses were finally approved; and

h. Making other misrepresentations detailed herein.

480. As described herein in paragraphs 1–308, Product Defendants knew such representations were false and that the truth was unknown and undiscoverable to Dr. Schubert and Plaintiff.

481. Product Defendants knew or should have known that, due to the life-preserving nature of Fabrazyme® and due to patients' reliance on Fabrazyme® to treat a deadly disease, that actions affecting, and misrepresentations regarding, the supply and efficacy of Fabrazyme® would have great influence over patients and their family members, including Dr. Schubert and Plaintiff.

482. As a direct and proximate result of such repeated actions, Dr. Schubert and Plaintiff suffered severe emotional distress, which distress caused Dr. Schubert to suffer the

physical harms and/or aggravated the physical harms detailed herein, and Plaintiff to suffer extreme loss of energy, inability to sleep, loss of appetite, loss of ability to perform routine activities, loss of ability to focus, loss of sleep, and loss of overall health.

483. As a result of these injuries, death, and conditions, Plaintiff has suffered damages as set forth herein.

EIGHTEENTH CLAIM FOR RELIEF
(Intentional Infliction of Emotional Distress v. Product Defendants)

484. Plaintiff incorporates all preceding paragraphs and further alleges as follows:

485. Product Defendants promote Fabrazyme® as a life-preserving drug.

486. As described herein, Dr. Schubert and Plaintiff relied on Product Defendants' representations that Fabrazyme® was a life-preserving drug.

487. Product Defendants knew or should have known that, due to the life-preserving nature of Fabrazyme® and due to patients' reliance on Fabrazyme® to treat a deadly disease, that actions affecting, and misrepresentations regarding, the supply and efficacy of Fabrazyme® would have great influence over patients and their family members, including Dr. Schubert and Plaintiff.

488. Because Product Defendants affirmatively decided to sell patients, including Dr. Schubert, Fabrazyme® at a dose 70% lower than was approved by the FDA, Product Defendants assumed a duty to provide complete and accurate information to physicians and patients regarding the dose, its risks, and the duration with which such dose would be provided, and also duty to provide a drug which was safe and efficacious.

489. Product Defendants' duties included, but were not limited to, a duty to warn that (a) the dose of 0.3 mg/kg of Fabrazyme® would be insufficient to prevent accelerated and, in

some cases, potentially catastrophic clinical deterioration in many patients, (b) male patients with significant cardiac disease, such as Dr. Schubert, were particularly likely to suffer accelerated medical decline if they skipped doses or took reduced doses, (c) if such a patient showed signs of medical decline on the reduced dose, that such decline would, in many cases, be irreversible even if full dose was eventually restored, (d) the Vedder study was not reassuring as to the clinical efficacy of a 0.3 mg/kg dose of Fabrazyme®, and (e) the Lubanda study was neither assuring nor clinically relevant on this issue.

490. Product Defendants intentionally caused Dr. Schubert and Plaintiff severe emotional distress by:

a. Intentionally and/or recklessly causing a Fabrazyme® shortage;

b. Intentionally and repeatedly misrepresenting to Dr. Schubert and Plaintiff the length of the shortage, causing Dr. Schubert and Mrs. Schubert to plan medical decisions around a resupply of full dose Fabrazyme® which did not occur;

c. Intentionally misrepresenting to Dr. Schubert that if he and other patients skipped doses of Fabrazyme® in August and September, 2009, a further reduction in dose could be avoided;

d. Intentionally misrepresenting the efficacy of Fabrazyme® at the FDA-approved dose;

e. Intentionally misrepresenting the efficacy of Fabrazyme® at doses lower than the FDA-approved dose;

f. Intentionally failing to timely respond to emergency requests for Fabrazyme® after Dr. Schubert's health began to deteriorate on reduced doses;

g. Intentionally failing to timely ship and supply Fabrazyme® to Dr. Schubert in February, 2010 after additional doses were finally approved; and

h. Intentionally making other misrepresentations detailed herein.

491. As described herein in paragraphs 1–308, Product Defendants knew such representations were false and that the truth was unknown and undiscoverable to Dr. Schubert and Plaintiff.

492. As a direct and proximate result of such repeated actions, Dr. Schubert and Plaintiff suffered severe emotional distress, which distress caused Dr. Schubert to suffer the physical harms and/or aggravated the physical harms detailed herein, and Plaintiff to suffer extreme loss of energy, inability to sleep, loss of appetite, loss of ability to perform routine activities, loss of ability to focus, loss of sleep, and loss of overall health.

493. As a result of these injuries, death, and conditions, Plaintiff has suffered damages as set forth herein.

PRAYER

WHEREFORE, Plaintiff prays for a judgment against Defendants in an amount to be determined by the trier of fact for the following damages:

1. For economic damages in an amount to be alleged and proven at trial;

2. For non-economic damages in an amount to be alleged and proven at trial;

3. For consequential damages, in an amount to be alleged and proven at trial;

4. For punitive damages; and

5. For any other further legal and/or equitable relief deemed just and proper by the court, including, without limitation, attorney fees, costs, pre- and post-judgment interest, and any other damages recoverable.

JURY DEMAND

Plaintiff demands a jury trial of all issues of fact in this matter.

DATED this 29th day of January, 2015.

EISENBERG GILCHRIST & CUTT

/s/ Jeffrey D. Eisenberg
Jeffrey D. Eisenberg
Jordan P. Kendell
Jeff M. Sbaih

Plaintiff's Address:
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